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ABSTRACT

The Hemophilia Health Education Planning Project was designed to (1) create a set of tools useful in hemophilia planning and education, and (2) create a planning model for other diseases with similar factors. The project used the game-simulations technique which was felt to be particularly applicable to hemophilia health problems, since as a planning tool, games offer an opportunity to examine alternative forms of social organization, resource allocation, and distribution of decisionmaking power. A brief description of the project is presented in two major sections, Hemophilia Health Care Planning and Education: A Gaming Approach, and The Planning Process in Perspective: Major Recommendations. Appendix A, representing the major part of the document (55 pages), presents the complete record of the project proceedings, and is categorized into three parts: Hemophilia Health Planning Project--Project Design Protocol; Hemophilia Planning Meetings; and Outline of Planning Sessions, the largest part, which is a slightly condensed description of each of the hemophilia games, with full instructions given to the players, and the players' responses. Appendix B is a five-page glossary of terms. (HD)

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THE HEMOPHILIA GAMES:

AN EXPERIMENT IN HEALTH EDUCATION PLANNING

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National Heart and Lung Institute

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TABLE OF CONTENTS

page

Preface. v

Project Members. vi

Section One

Hemophilia Health Care Planning and Education:
A Gaming Approach. 1
Introduction. 1
Gaming Simulation and Planning--Background. 1
The Health Problem--Hemophilia. 3
Process Overview: The Planning Game. 4

Section Two

The Planning Process in Perspective:
Major Recommendations. 7
Introduction. 9
A Review of the Planning Process. 9
The Planning Sessions 9
The Gaming Sessions 10
Care Sector 10
Product Sector. 11
Payment Sector. 12
Education and Research Sector 13
Recommendations 14
Establishment of a National Hemophilia
Coordinating Body 14
Design and Implementation of a System of
Comprehensive Care Centers. 15
Other Specific Action Recommendations 15

Appendix A

Record of Proceedings. 19
Part One - Hemophilia Health Education Planning
Project: Project Disign Protocol 22
Introduction and Special Considerations 22
Planning Design 22
Other Possible Outcomes 24

page

Part Two - Hemophilia Planning Meetings:
October 14, 1974 and February 3, 1975. 25
Program Goals and Directions - The Treatment of Hemophilia (Interim Statement). 25
Program Development. 26
Activity A - Creation of a National Hemophilia Care Quality Review Board. 29
Activity B - Data Collection and Evaluation. 30
Activity C - Payment and Funding Rationale 30
Activity D - Protocol to Standardize Centers 31
Activity E - Evaluation of Existent Facilities . . .31
Activity F - Development of Pilot Centers. 31
Activity G - Satellite Programs. 32
Sequencing of Plan Development 33
Issues to be Discussed 34
The Next Steps 34

Part Three - Hemophilia Games:
Scenarios and Responses. 41
Session I. 41
I. Instruction of Players 41
II. Presentation of Scenario 1 42
III. Instructions on Questions to be Addressed and Statements to be Prepared. 45
A. Catastrophic Disease National Health Insurance Act. 46
B. Consequence A: 13,000 Additional Hemophiliacs Entering the System 51
C. Consequence B: $5,000 Ceiling on Hemophilia Care Per Individual 59
Session II 61
I. Instruction of Players 61
II. Presentation of Scenario 2 62
A. Establishment of 15 Regional Centers . . .63
B. Consequence A: Five Centers Named to Carry on Hemophilia Research 68
C. Consequence B: National Hemophilia Centers to Become Depots for Blood Products 70

Appendix B

Glossary of Selected Terms. 77

PREFACE

The Hemophilia Health Education Planning Project (HHEPP) was developed jointly by the Office of Prevention, Control, and Education, the Division of Blood Diseases and Blood Resources (both of the National Heart and Lung Institute), and the National Hemophilia Foundation. The project began as a research effort to design an appropriate education program for the hemophiliac, his family, the general public, and the health professional. What at first appeared to be a relatively simple and straightforward task, quickly proved complex. The design of any educational program depends ultimately on its goals and the context in which the education might be delivered. Too often health education programs are simply "add-ons" to pre-existent health care endeavors and have not been carefully integrated into the purpose and design of the overall enterprise. The net effect is that education programs are often irrelevant, and sometimes counter-productive.

Problems began with the HHEPP because neither the operative goals nor the context (system of care) were clear. To proceed, we had to give substantial effort to identifying (and, as it turned out, developing) the hemophilia care system, and to defining goals. Early in our efforts with hemophilia education, we recognized not only the obligation but also the opportunity to develop and test a planning procedure that would delineate the structure of the care system and allow the development of an educational program appropriate to that system. However, planning was problematic because of several factors:

- Sparse and inaccurate data.
- Interlocking systems, i.e., the hemophilia care system and the blood distribution system.
- Imponderable future events such as the enactment of National Health Insurance.

Faced with these difficulties, we hypothesized that a planning process utilizing *gaming* might enable us to simulate *future* events and make *present* judgments about appropriate courses of action.

The situation encountered by the Hemophilia Health Education Planning Project is not unique, and our experience might help others. Therefore, in the following material we have fully outlined our process and rationale, our deliberations, and our conclusions. From the beginning, we considered our efforts experimental. For the participants, the project was apparently satisfying and informative, and therefore successful. The long-term impact of our work remains to be seen.

This document is long because we have written it for several audiences. Individuals primarily concerned with the care of the hemophiliac may want to start with the "Summary and Recommendations" and work backwards to determine how we arrived at them. Planners and staff of health care systems may want to read the whole document. Gaming enthusiasts will find a theoretical description in the first section. A judicious use of the index will be helpful to all readers.

In conclusion, we must emphasize the experimental nature of our work. Our approach held promise, but not certainty. Any success is attributable to the enthusiasm and dedication of the participants; their investment of time and effort was substantial. Also, our heartfelt thanks go to our "gamers" and consultants. This document is the product of all participants and consultants, and special credit goes to Drs. Cathy Greenblat and John Gagnon for Section I and to Ms. Caroline Petit for the rest.

Harrison Owen
Project Officer

PROJECT MEMBERS

David Agle, M.D. (Care Sector)
Associate Professor
Department of Psychiatry
Case Western Reserve University Medical School
Assistant Physician
Director of Adult Psychiatry Outpatient Clinic
Director of Consultation Liaison
University Hospital of Cleveland
Member, Medical and Scientific Advisory Council
National Hemophilia Foundation
Member, Medical and Scientific Board of the Northern Ohio
Chapter of the National Hemophilia Foundation

Louis Aledort, M.D. (Care Sector)
Associate Professor of Medicine
Vice Chairman, Department of Medicine
Mount Sinai School of Medicine
Director of the Mount Sinai Comprehensive Care Hemophilia
Treatment Center, a World Federation of Hemophilia
designated International Training Center
Medical Director
National Hemophilia Foundation
Vice President
American Blood Commission

Jan van Eys, M.D. (Education and Research Sector)
Pediatrician and Professor of Pediatrics
Head of the Department of Pediatrics
M.D. Anderson Hospital, The Texas Medical Center
Member, Medical and Scientific Advisory Council
National Hemophilia Foundation
Member, Medical Advisory Board, Gulf Coast Chapter
National Hemophilia Foundation

John H. Gagnon, Ph.D. (Gaming Consultant)
Professor of Sociology
Department of Sociology, SUNY at Stony Brook

Marvin Gilbert, M.D. (Care Sector)
Assistant Clinical Professor
Department of Orthopedics, Mount Sinai School of Medicine
Medical Director
National Hemophilia Foundation
Director of the Mount Sinai Comprehensive Care Hemophilia
Treatment Center, a World Federation of Hemophilia
designated International Training Center

Cathy Greenblat, Ph.D. (Gaming Consultant)
Associate Professor of Sociology
Douglass College, Rutgers State University
The State University of New Jersey

John Grupenhoff, Ph.D. (Payment Sector)
President
Science and Health Communications Group
Legislative Representative
National Hemophilia Foundation

Leon W. Hoyer, M.D. (Care Sector)
Professor of Medicine
Head, Hematology Division
Department of Medicine
University of Connecticut Health Center
Chairman, Medical Advisory Board for the Connecticut
Chapter of the National Hemophilia Foundation

Aaron Josephson, M.D. (Product Sector)
Associate Director of Clinical Research
Hyland Laboratories
Division of Travenol Laboratories, Inc.
Member, Blood Resources Committee
National Hemophilia Foundation
Member, Regionalization Task Force
American Blood Commission

Aaron Kellner, M.D. (Product Sector)
Executive Vice President, Director
Greater New York Blood Program
Clinical Professor of Pathology
New York Hospital, Cornell Medical Center

Harvey Klein, M.D. (Payment Sector, Education and Research Sector)
Assistant to the Director
Division of Blood Diseases and Resources
National Heart and Lung Institute

Don Meyers (Payment Sector)
Assistant Vice President of Institutional Affairs
Blue Cross/Blue Shield of Greater New York
Member, Blood Resources Committee
National Hemophilia Foundation
Member, American Blood Commission

Ian A. Mitchell, M.D. (Product Sector)
Office of Special Health Projects
Office of the Assistant Secretary for Health
Department of Health, Education and Welfare

Harrison Owen (Project Officer)
Program Director
Office of Prevention, Control and Education
National Heart and Lung Institute

Caroline Petit, Sc.M. (Consultant)
National Heart and Lung Institute

Paul Reisel (Payment Sector)
Chief, Medical Services Reimbursement Branch
Bureau of Health Insurance
Social Security Administration

Ernest Simon, M.D. (Education and Research Sector)
Director, Division of Blood Diseases and Resources
National Heart and Lung Institute

George Theobald (Education and Research Sector)
Executive Director
National Hemophilia Foundation

Section One

HEMOPHILIA HEALTH CARE PLANNING AND EDUCATION: A GAMING APPROACH

by

John Gagnon, Ph.D.
Cathy Greenblat, Ph.D.

Section One

HEMOPHILIA HEALTH CARE PLANNING AND EDUCATION: A GAMING APPROACH

INTRODUCTION

The gaming project began in September 1974 and ended in June 1975 under the official title of the Hemophilia Pilot Planning Project. It was jointly sponsored by the Office of Prevention, Control, and Education (OPCE), the Division of Blood Diseases and Resources (DBDR) of the National Heart and Lung Institute (NHLI), and the national offices of the National Hemophilia Foundation. The project was designed for hemophilia, but was also intended to have broader implications for the work of the NHLI. There were two major objectives of the project: (1) to create a set of tools useful in hemophilia planning and education, and (2) to create a planning model for other diseases with similar factors. Gaming simulation is particularly useful for program planning when both manifest and latent conflicts of interest occur among the members of the social system or organization that is the subject of the planning. Because a number of nonquantifiable factors, such as self-interests and group interactions, created difficulties in using traditional planning procedures for hemophilia health care, gaming was used in the project.

It was hoped that some of the data from the planning games might be usable in developing materials for school curricula (elementary grades to medical school classes) concerned with the medical care delivery system. The original document of intent thus included the following statement: "Assuming that the process outlined above runs to completion, it is reasonable to expect general side benefits in terms of educational packages which might be used in situations as disparate as high school social studies and graduate courses in health care administration..." In fact, such a game was developed and will be published separately under the title of *"Blood Money."*

GAMING SIMULATION AND PLANNING--BACKGROUND

A simulation is a dynamic model of the central features of a system. It not only exposes the state of a system at any given moment, but also demonstrates the processes by which the system changes. To understand the power of a simulation, we can contrast the static model of the solar system created by students in a high school science class--a set of fixed balls of various sizes set at various distances--with the operating model of the same system in a planetarium. The planetarium display offers a look at the changing characteristics of the solar system

through time, and by incorporating more features of the system, it provides more complex information than the set of fixed balls about the dynamic properties of the planetary system.

Such a simulation abstracts the essential elements and processes of the real-world system, and then creates a simplified, abstract model to represent the system in a more immediately understandable way. Some aspects of the system are disregarded either because they make little difference in the real world or for the purposes of the simulation. As with all abstract constructions of the world, the usefulness of the simulation depends upon selecting particular features and upon making them salient. The same system may be modeled in different ways for different purposes; hence, a road map, a surveyor's map, and a real-estate zoning map of the same region will all be different. The features selected for simulation will depend upon its purpose.

Simulations can operate in several ways. In some situations the simulation may involve only a computer which plays through a combination of previously programmed elements; in "person-machine" simulations, persons and computers are mixed and the decisions of both influence the outcomes; and finally, in "all-person" simulations, all of the roles and the calculations of consequences are managed by the human participants.

Conventionally, the "person-machine" and the "all-person" simulations are referred to as "game-simulations," "gaming-simulations," or "simulation games." The term "game" is applied to those simulations involving human decisions because the environment and activities of the participants have the characteristics of games: players have *goals*, sets of *activities* to perform, *constraints* on what can be done, and pay-offs (both good and bad) which are the *consequences* of their actions. Since the gaming-simulation is patterned on real life--that is, the roles, goals, activities, constraints, consequences, and the linkages among these elements simulate the real world--participants experience a social situation but in a game-like way. Therefore, although the players can play the games seriously, they are not in a real-risk situation. The novice pilot in the simulator "plays at" landing the aircraft, but he also takes it seriously because eventually he will have real responsibility for life and property. The gaming simulation highlights the important processes of a system at the same time that it offers the players non-risk exposure to new roles, new activities, new constraints, and new consequences.

Players can experience these novelties in play-time, a compressed or expanded time frame. Gaming simulations reduce a set of complex elements and processes to a manageable size. By collapsing time and space, they permit rapid comprehension of the fundamental principles of the situation being simulated.

Also, a simulation can reveal the underlying sociological conflicts of a situation because the players are actively involved in cooperative and conflicting activities. They can begin to see how covert and overt commonality of purpose or disparity of interests can influence decisions, and how various decisions can affect the system as a whole.

Gaming is important and useful since it offers an active, synthetic approach to learning, communication, and planning. It is *active* because the players explore environments; look for clues to system constraints, opportunities, rewards, and punishments; interact with others; and, through participation, modify their perceptions, responses, and strategies. It is *synthetic* in that it provides an opportunity to understand many aspects of a social system and the interrelatedness of decisions made within it.

As a planning tool, games offer an opportunity to examine alternative forms of social organization, resource allocation, and distribution of decision-making power as a test of the efficacy of different ideas, the costs and rewards of various options, and the ease or difficulty of moving from the present to some desired future state. Possible futures can be explored in microenvironments.

THE HEALTH PROBLEM--HEMOPHILIA

Hemophilia is a hereditary disorder in which the patient's blood clots very slowly or not at all, leading to uncontrolled bleeding during injury or surgery unless the appropriate clotting factor (Factor VIII or Factor IX) is supplied promptly. External bleeding from small wounds is less of a problem than internal hemorrhaging into joints.

Presently, there are between 25,000 and 30,000 hemophiliacs in the United States. Although this number is not large, hemophilia does constitute a major health problem for several reasons. First, treatment must be continued throughout the patient's lifetime at enormous costs; the alternatives are disablement and death. The disease could be controlled effectively by prophylactic care, but such therapy would cost between $50,000 and $60,000 per patient annually. Second, treatment of hemophilia places a large demand on the nation's blood supply. Even if a payment mechanism were available through a National Health Insurance, the costs would still be great, and such massive prophylactic treatment would severely affect the national blood supply. Third, the social, psychological, and economic impact of hemophilia on the patient and his family is immense and serious.

Medical management of hemophilia has progressed in the past two decades, but complete data on the dimensions of the health problem and the delivery system are lacking and should be collected. A central aim of this project was to develop a better understanding of the hemophilia care system and its operation.

PROCESS OVERVIEW: THE PLANNING GAME

There were three components to the overall project: (1) two planning sessions, (2) preparation of a Fact Book, and (3) designing and playing the planning games.

1. Two Planning Sessions

The initial planning of the project emerged from two preliminary all-day sessions of the Hemophilia Education Planning Group, composed of all staff and participants listed on pages ii-iv of this report. The meetings were held at Mount Sinai Hospital in New York City in October 1974 and February 1975. Minutes of both meetings were sent to all participants, and between the two sessions a short article describing the rationale and nature of gaming was sent.

The agenda for both meetings was as follows:

a. Introduction of the planning procedure and participants.

b. Goal setting. The group was asked to establish tentative five- and ten-year goals for the care of the hemophiliac patient. Individuals formulated goals privately at first; subsequently, these goals were discussed and debated by the group as a whole. It was emphasized that these goals were only preliminary statements and were therefore open to constant revision during the planning process.

c. Flow chart. Given the stated goals, an attempt was made to construct a logical and coherent chain of events which would yield those outcomes (known or hoped for).

d. Summary and preliminary definition of areas for gaming. The flow chart was analyzed (1) for unanswered, substantive questions, and (2) for highly ambiguous areas for which gaming might be used.

2. Preparation of a Fact Book

After these two meetings, a comprehensive Fact Book was prepared by Ms. Caroline Petit, based upon many resources and contacts. The purpose of the Fact Book was to describe the current state of the hemophilia care system, to recognize issues, and to point out the lacks in our knowledge about hemophilia care; thus, its purpose was not to collect new data but rather to gather and synthesize existing information and opinions. This 65-page document was sent to participants before the first gaming session with sufficient time to permit them to study it in advance.

3. Design and Play of the Planning Games

Issues and scenarios. On the basis of the information revealed in the preliminary sessions and in the Fact Book, a number of events and issues were considered for gaming. The two main points of concern expressed by the participants were the introduction of National Health Insurance and the development of comprehensive care centers for the treatment of hemophiliacs. Both were highly desired, but the impacts were ambiguously defined in the discussions. Hence, six "perturbations" involving these two issues were developed and scenarios were written.

The general character of the participants' task was described to them as follows:

> "Over the course of the next few hours, and then continuing the next time we meet, we are going to act as if some events that seem likely future developments have actually occurred. Some of these may seem extremely fortuitous events with positive consequences for those concerned with hemophilia care, whereas others will seem less promising, perhaps even highly threatening to your interests and conerns. We may assume that all will prove to have mixed characters upon further exploration. Our concern is to develop a better understanding of the impact such events would have on the hemophilia care system as it exists and as it is likely to develop over the next decade."

> "As an attempt to 'tease out' some of the impacts, we will simulate the initiation of the event by telling you what has happened and will ask you to work in groups to develop responses to the event to help determine some of the less obvious dimensions of the system and its stress points. Thus, your thoughtful responses to the events should help reveal where work is needed to make those things you desire come about, and where work may be needed to prevent other consequences that emerge in the simulation from happening in reality."

> As the issues or events were presented, participants were asked to formulate group statements in response to three questions:

- HOW WILL THIS EVENT AFFECT YOU?
- WHAT WILL YOU DO OR TRY TO BECAUSE OF IT? (i.e., How will you alter your behavior, operations, etc.?)
- WHAT SHOULD OTHERS IN THE SYSTEM DO ABOUT IT?

Role Specification and Allocation. Players were divided into four groups representing the four sectors of the system identified in the preliminary session: Care, Product (blood), Payment, and Research and Education. They were urged to play themselves, with their own values,

knowledge, interests, and political views. They were asked, furthermore, to broaden their perspectives to consider the impacts on their sector (e.g., Care) as a *whole*--that is, to speak both as a representative of the sector and as an individual.

The Games in Operation. In actual operation, the games sought to generate the highest level of "reality" possible. Once the initial and scenario had been presented, the participants began to plan strategy and to bargain with their co-players in order to create a meaningful and workable response from the point of view of their group's sector (Care, Product, Payment, Research and Education). At times these interchanges became fairly heated, and it was in the pressure of exchange that the positions and working strategies of the various groups came to light. The general atmosphere more nearly resembled a negotiating session than a closely reasoned discussion. There are advantages and disadvantages to this approach. On the negative side is a certain lack of clarity and fine detail. On the positive side, the participants appeared to respond as they might under "real" pressure and not as they *thought* they *would* or *should* respond.

During the sessions, the various interchanges were recorded and summarized, with not only the content but also the interactions noted. This material is reproduced in Appendix A. The reader is urged to peruse this material, for no description of the games can illustrate the method and process as effectively as the proceedings can.

After each gaming session, an edited transcript of the proceedings was sent to all participants to provide an opportunity for more reasoned reflection than was possible during the games.

Preparation of Recommendations and the Final Report. Prior to the last meeting of the planning sequence (June 17, 1975), the entire process was summarized. A draft of recommendations was carefully debated, and several recommendations were reworked by the entire participant group. There was essentially no disagreement about recommendations of a substantive nature, which is noteworthy considering the disparate nature of the group. The recommendations form Section Two of this report. The entire report, as presented here, was then prepared by NHLI staff and consultants and circulated to all participants for editorial comments.

Section Two

THE PLANNING PROCESS IN PERSPECTIVE: MAJOR RECOMMENDATIONS

Section Two

THE PLANNING PROCESS IN PERSPECTIVE: MAJOR RECOMMENDATIONS

INTRODUCTION

The Hemophilia Health Education Planning Project was initiated to explore the implications of foreseeable changes in the health care system on the delivery of hemophilia care and how these would affect education programs. The planning process used in this project, and explained in the preceeding section, described the overall goals of the project and helped us identify data needs and the conflicts among competing interest groups. One can view this planning process as a series of steps. What follows is a summary of the planning sessions and our conclusions and recommendations.

In 1971, a study by the National Heart and Lung Institute estimated that if comprehensive care were extended to all symptomatic hemophiliacs, 3 to 8.7 million units of plasma annually would be required just to treat episodic bleeding. If all severe hemophiliacs were maintained on a prophylactic program of cryoprecipitate, 13.5 million units of whole blood would be needed to prepare this Factor VIII concentrate. Currently, about 2.2 million liters of plasma and 8.0 million units of whole blood are collected annually. Because NHLI is charged with studying all aspects of managing the country's blood resources, this potential demand for blood products is of vital concern to the Institute.

A REVIEW OF THE PLANNING PROCESS

The Planning Sessions

Participants decided that the primary goal of hemophilia care should be physical freedom for the hemophiliac patient. Physical freedom could best be achieved by adequate home therapy and by the repair and prevention of physical deformity. To attain this goal, a number of objectives were identified:

1. The evaluation, improvement, and standardization of treatment, facilities, and blood products.

2. An epidemiologic study of the distribution of patients and the severity of their disease, and the establishment of a Hemophilia Registry.

3. Further research on the prevalence, risk, and effective management of inhibitors.

4. An education/information program for patients, health care professionals, and the public to create informed participation and support of treatment programs.

5. A strengthened organization, primarily through the National Hemophilia Foundation, to provide personal and group support for patients.

These objectives, it was assumed, could best be implemented through a national network of comprehensive care centers supported by an interconnecting network of satellite centers. These comprehensive care centers would be evenly distributed across the country, covering 50% of the hemophilic population within five years and 100% in ten years. The participants further assumed that universal entitlement, most likely National Health Insurance, might well occur in the future. National Health Insurance could be the catalyst for comprehensive care centers. With National Health Insurance, payment for medical services most likely would to to a care center and not to the individual physician or patient. Another necessary prerequisite for comprehensive care centers would be a well-organized blood supply system to minimize problems of distribution and cost.

During the second planning session, the participants acknowledged the necessity of creating an advisory/regulatory commission (a Hemophilia Commission) to prepare the way for establishing (1) a coordinated network of comprehensive care centers, (2) a uniform payment system, (3) a distribution system for blood products at a reasonable price, and (4) standards for evaluating health care delivery.

The Gaming Sessions

The players were divided into four groups representing the four sectors of hemophilia care: Care, Product (blood), Payment, and Research and Education. Their points of view are summarized below.

Care Sector. In the gaming sessions, the Care Sector defined some of the functions of a Hemophilia Commission. Members felt that the Commission should be similar to the board proposed in the bill H.R.I., which is concerned with the type of care delivered, the qualifications of providers, and determination of reasonable reimbursement charges for a service. The group recommended that the Commission endorse a group-provider approach to hemophilia care and that it de-emphasize single-physician care and prophylaxis. The planners recognized that a Hemophilia Commission would have to work closely with the American Blood Commission (ABC) because hemophilia care depends directly on the blood supply system. Working with the ABC, the Commission's first official act would be to set standards of care.

The question then arose, "What is the best care?" The answer is not known. The care group therefore recommended that clinical trials on prophylaxis and home care be started at once to answer that question, and that the Commission set standards and monitor care. When model comprehensive care centers are instituted, the centers would become the yardstick against which the Commission would measure hemophilia care throughout the country.

An NHLI study found that often a physician is treating only one hemophiliac. Some group members questioned whether adequate care can be delivered by a physician having limited experience with this disorder. No agreement was reached as to whether the Commission should monitor individual physician care.

The Care Sector's response to National Health Insurance was that providers would be overwhelmed. Currently, most programs offering comprehensive care have sparse staff and facilities. With universal entitlement, daily operation and centralization of services would become imperative, but difficult to achieve.

To create a system of comprehensive care centers, initial capital outlay and early operating money must be found. Hemophilia care is specialty care that requires a base population before it can be offered. At first, this base population may not be present, and there would be no guarantee that enough hemophiliacs would use these centers to justify offering specialty services. Operating funds would be needed to allow the centers to function with a complete staff before there were adequate numbers of patients.

The Care Sector believed that there is a need for a satellite program. The satellite program would be smaller centers that had been carefully evaluated by the larger, complete comprehensive centers to determine whether primary or secondary care should be provided by the satellites. A satellite could consist of one private physician with a limited number of hemophilia patients. Satellite staff could be educated and care could be monitored by closed-circuit television from the parent center to the satellite. As an enticement for satellite centers, money should be made available to upgrade care and services.

Product Sector. In the planning sessions, the Product Sector felt that if the ABC developed as expected, blood products would be manufactured and distributed within a rational system. The consensus was that a Hemophilia Commission ought to be delegated a review function by the ABC to oversee the use of blood products required by hemophiliacs.

In the gaming sessions, it was clear that much more must be known about the use of blood products before any rational system for production

and distribution could be devised. How much product is required for each level of care--crisis, home care, prophylaxis, and surgery? Data on demand are lacking. The group recommended that collection of data on blood product usage should begin as soon as possible with the ABC acting as the data collection body. Insufficient data, as the gaming exercise demonstrated, could result in unnecessary expansion by the pharmaceutical industry.

The product group recommended that hemophilia centers be associated with regional planning networks, such as regional blood centers and regional medical programs. Regional blood centers would have expertise in storing, managing, and collecting data on blood, and this expertise should be utilized. If regional blood centers are created, the players decided, blood products for hemophiliacs should be distributed through these blood centers to the homophilia centers and not to individual physicians or pharmacies. Some participants wanted to exercise more control over product distribution.

The suggestion was made that 25 national leaders in hemophilia care be assembled and that they achieve a 95% agreement on care under most circumstances. A list of questions based on the 95% agreement would be administered to physicians treating fewer than three hemophiliacs. This questionnaire could serve as a continuing education qualifying requirement.

Gaming suggested that the demand for product would increase in the event of National Health Insurance and/or the establishment of comprehensive care centers nationally. Physicians would have to be prevailed upon to use the most efficient product. The cost of Factor VIII and Factor IX concentrates would rise as the demand for other fractionated blood products decreased proportionately because more of the cost for fractionation would be assigned to hemophilia blood products. To counteract this rising cost of antihemophilic factor, it was recommended that a government fractionation center be established as a model. This center could become the recipient of pooled plasma obtained from regional blood centers as directed by the ABC. These products, priced at cost, would be the competition for the pharmaceutical industry's blood products.

Payment Sector. In the planning sessions, it was assumed that single source funding (National Health Insurance) would lead to a more centralized system of hemophilia care. In the gaming sessions, it was realized that the reverse very probably would occur; hemophilia care would become profitable and physicians would compete for hemophilia patients. Consequently, the payment group devised different strategies to encourage patients to use centers, which would be regulated to provide optimal care.

First, using the Bureau of Health Insurance in the Social Security Administration as a third party payer, there would be a two-tier payment system. Noninstitutional providers, physicians, and pharmacists would be reimbursed for the actual cost of care--the audited cost. The patient's money should go farther in a center because centers would be able to practice economies of scale. If regional blood centers distributed products, then third party payers would also be able to reimburse them their full costs. The regional blood centers' costs would be the actual, audited cost for product. The payment system also would be designed to include research as part of the audited cost of care.

If this two-tier payment plan is not sufficient inducement for patients, it might be possible to mandate a care package or to develop a reimbursement plan which would only pay for care obtained from an approved program.

To compare care systems, the payment group advised gradual institution of a center system. At the beginning, there would be two different systems with enough centers to provide alternatives to existing care. Data would be accumulated on the cost and quality of care of the two systems to ascertain if one system is more efficient.

Education and Research Sector. In the planning sessions, it was considered essential to have an education/information program to ensure participation by informed patients and health professionals and to foster public support. Patient involvement in the centers program was to be further strengthened through the National Hemophilia Foundation, the patient organization for hemophiliacs.

During gaming the Education and Research Sector proposed that, to ensure an education/information program component of the center system, an educational director at each center should be responsible for continuing education for the health professional and paraprofessional. Lay and patient education would be a cooperative venture of the centers and the local National Hemophilia Foundation chapters.

To increase patient involvement in planning for care, patient representation on the Hemophilia Commission was recommended. Players were concerned that patients' rights as consumers to seek or not to seek care must be respected, and the Commission would have to balance the rights of a hemophiliac against the rights of society which has to bear the burden if he becomes debilitated and unproductive because he did not seek care.

During the gaming session on National Health Insurance, the National Hemophilia Foundation's response was to modify its activities, becoming

a consumer protection agency for all blood diseases groups. It would design information programs to inform this new constituency about which care was quality care. The Foundation should seek a precise legal definition of hemophilia in order to identify the patient population more closely.

Prior to the establishment of centers, the Foundation might request a grant to determine who would use the centers and to measure the increase in demand for such services. After establishment of the centers, the National Hemophilia Foundation would continue its casefinding functions. It would also support efforts to increase coordination among centers, assist in standardizing nomenclature, and set guidelines to prevent overlapping research and other activities.

During gaming the group made some policy suggestions about research: (1) more basic research should be supported than clinical research; (2) research into blood products should be conducted by regional blood authorities, if they are created; and (3) all research money should be awarded competitively on the basis of a project's merits.

Concern was also expressed that because hemophilia care is experimental, established standards must permit wide latitude in care and center operation.

RECOMMENDATIONS

At the final sessions, the participants summarized their findings and made the following recommendations.

Establishment of a National Hemophilia Coordinating Body

Planning for hemophilia care today cannot be based only on hoped for events of tomorrow. Even if National Health Insurance were enacted tomorrow, it would not be operational until 1980. At least two years would be devoted to planning its implementation. Then, implementation must be gradual so as not to disrupt the country's health care system. A plan for hemophilia care must offer services that can be accomplished now.

Hemophilia care has to be centralized because (1) hemophilia is a rare disease and hemophiliacs are dispersed throughout the country; and (2) hemophilia requires specialty care; a highly trained, multi-specialty, closely coordinated staff; and a sophisticated laboratory. The centralization of hemophilia care will have to occur without the catalyst of National Health Insurance. Gaming demonstrated that universal entitlement could lead to competition among private physicians for hemophilia patients instead of effecting increased centralization. Therefore,

THE HEMOPHILIA HEALTH EDUCATION PLANNING GROUP RECOMMENDS THAT THE FIRST STEP TOWARD ORGANIZING HEMOPHILIA CARE BE THE CREATION OF A NATIONAL HEMOPHILIA COORDINATING BODY.

Such a coordinating body may be established (a) as a National Hemophilia Commission, by executive order, or (b) as a standing committee of the American Blood Commission with special responsibility for hemophilia.

The majority of the planning group favored a National Hemophilia Commission; but, in either case, the coordinating body must possess real authority to affect the flow of payment and product as these elements relate to the needs of and quality of care for the hemophiliac.

Design and Implementation of a System of Comprehensive Care Centers

The primary function of the Hemophilia Coordinating Body would be to design and implement a national system of comprehensive care centers. Although several modes for the delivery of care may be appropriate, the planning group concluded that the focal point for these modes should be a system of comprehensive care centers. Through these centers and related satellites, critical and scarce health manpower and blood resources could be carefully managed. Many details remain to be clarified; however, the general shape of the planning group's concept may be seen in the section of this report describing the initial planning sessions (Appendix A, Part II).

Other Specific Action Recommendations

In the development of a functional center concept (or conceivably the development of alternatives), the planning group recommends that the Hemophilia Coordinating Body concentrate its initial effort in the following six areas.

I. Data Collection

A. Collect data on product use.

1. Estimate product use for every level of treatment--crisis, home care, prophylaxis, surgery, and moderate and severe hemophilia.

2. Construct a profile of current product use and proposed product use.

3. Estimate how much whole blood is fractionated.

4. Estimate how much plasma is fractionated for anti-hemophilic products.

5. Determine how many plants have the capacity to fractionate.

B. Initiate an epidemiological study on patient distribution and condition.

C. Establish a registry of hemophilia patients.

D. Determine present availability of and future needs for psychosocial services.

II. Evaluation of Care Systems

A. Examine the organizational structure and delivery of care in hemophilia centers and other categorical disease centers in all major areas including psychosocial evaluation and treatment.

B. Compare the delivery of care in hemophilia and other disease centers to that delivered by private physicians.

C. Design studies to evaluate the care delivery capacity and organizational structure of these centers.

III. Education

A. Design studies to determine how patients learn about their disease.

B. Design studies to identify current educational programs to evaluate their effectiveness, and to assess changes in these programs that patients and professionals would like to make.

C. Explore ways to educate the individual who is delivering care.

D. Explore ways to educate families about new treatment modes and about how they can cope with the psychosocial problems of hemophilia.

E. Explore ways to make the public more aware of the physical capabilities of hemophiliacs.

IV. Research

Request that clinical trials on prophylaxis be started by the National Institutes of Health.

V. Fractionation Center

- Study the need for and the feasibility of establishing a Federally funded fractionation center. Questions that should be answered include:

  -How many plants do or do not produce AHF?
  -What is the pricing structure?
  -What is the American Red Cross planning to do in this area?

VI. Model Building

A. Develop standards of care delivery based on the above data gathering. In effect, these standards would become models for hemophilia treatment.

B. Solicit proposals from existing centers to establish model centers. In metropolitan areas, existing centers could act as a consortium, initiating coordination among centers and reducing competition for patients and funds. Eventually, these consortia might be able to develop a centralized product procurement and payment system for hemophilia care similar, perhaps, to a Health Maintenance Organization.

Appendix A

RECORD OF PROCEEDINGS

Appendix A

RECORD OF PROCEEDINGS

The material in this section is essentially edited versions of the interim documents produced during the gaming process. We include them in their entirety for several reasons. First, we felt that the process we engaged in was almost as significant as the results we achieved. Indeed, we hope that a similar approach might be used in other areas of health care. With this thought in mind, we have tried to provide the reader with sufficient material to see not only what happened, but also *how* it happened. Second, much of this useful information does not fully appear in our Summary and Recommendations. Third, one of the most valuable aspects of this exercise was our increased awareness of the interactions between the various components of the care system under the different circumstances presented during the gaming process. The fact that certain alignments of self-interests or conflicts appeared should be borne in mind as the hemophilia care system evolves in the future.

This Appendix has three parts. The first, entitled "Hemophilia Health Education Planning Project," is the basic protocol of the goals and proposed operation. With some minor modification of dates, and a certain telescoping of activities, the process described was run to completion. The second part, entitled "Hemophilia Planning Meetings," describes the outcome of the two planning sessions held in October 1974 and February 1975, which outlined the basic goals for hemophilia care and designed a system to achieve these goals. Areas of ambiguity or uncertainty noted during these sessions formed the basis of the scenarios that were "gamed" in subsequent sessions. The last part, and the largest, is a slightly condensed description of each of the games, with full instructions given to the players, and the players' responses.

Note: Reference is made at several points to the Hemophilia Fact Book. This book is published under separate cover and is available upon request from the National Heart and Lung Institute under the title, Hemophilia, Hemophiliacs and the Health Care System, DHEW Publication No. (NIH) 76-871.

Part One

HEMOPHILIA HEALTH EDUCATION PLANNING PROJECT: PROJECT DESIGN PROTOCOL

INTRODUCTION AND SPECIAL CONSIDERATIONS

Effective planning for health education programs is a primary concern of the Office of Prevention, Control and Education (OPCE). Besides the typical areas of educational message, media, and technique, planning of future education programs must consider several essential factors: state-of-the-art research, strength of voluntary agencies, and existent and proposed legislation. The planning process is not simple, and there can be no guarantee of success.

Given the above considerations, OPCE is proposing a pilot project in cooperation with the National Hemophilia Foundation (NHF) and the Division of Blood Diseases and Resources (DBDR-NHLI) to test the effectiveness of a planning process designed by OPCE. This project will be conducted essentially as an in-house cooperative research program. Responsibility for the project will be shared equally by Mr. Harrison Owen (OPCE), Dr. Harvey Klein (DBDR), and Dr. Louis Aledort (NHF). It is stipulated in advance that each party involved in this project shall have the irrevocable right to unilaterally terminate the project at the conclusion of any one of the various phases enumerated below, if in his view the potential value of the project does not meet his expectations. It is further understood that this project is a pilot study which, although it concentrates on hemophilia, is intended to have broader applications. Therefore, all information about the conduct and development of the proposed planning process shall be available to NHLI as a whole. Confidentiality of any material shall be maintained only upon the specific request of a project participant.

PLANNING DESIGN

As presently conceived, the planning design is organized into five phases.

Phase I - Goal Setting

Schedule: Projected for late August or early September 1974 with five to six people from the Hemophilia Foundation plus representatives of OPCE and DBDR.

Objective: To establish concise goals for health care services for hemophiliacs on five- and ten-year timetables. These

goals may very well change during the course of planning, but they will nevertheless constitute a baseline for future efforts.

Phase II - Creation of a Data Book

Schedule: September-November 1974

Objective: To compile a Hemophilia Data Book comprising:

Numbers affected
Population description--age, race, location, economic level
Related diseases
Annual cost--treatment, job loss

Patient education needs
Professional education needs
Existent educational resources
Associated governmental agencies
Associated voluntary agencies
Existent legislation
Pending legislation
Related non-health organizations

It is not the objective of this phase to engage in any extensive *new* research. Essentially, we will compile available information. The purpose of this Data Book shall be to supply background information for the entire project.

Phase III - Creation of a Flow Chart

Schedule: December 1974, a one- to two-day session involving six to ten people from the Hemophilia Foundation plus representatives from OPCE and DBDR.

Objective: To arrange a timetable of events or activities which will positively or negatively influence the attainment of the described goals (Phase I), and to indicate events that we *think* will happen and those that we *hope* will happen. The net result of this exercise will be to describe the general parameters of activity for an overall hemophilia program. From this information, we would identify certain classes of activity: (1) things we definitely want to happen in situations where we are reasonably clear as to the potential results, and (2) things we are ambiguous about because of lack of clarity concerning potential results.

Items identified under #2 are potential candidates for gaming in Phase IV. Further refinement of issues will probably be necessary to clearly describe no more than three areas as subject matter for three games.

Phase IV - Gaming

Schedule: February-April 1975, three weekend gaming sessions with a maximum of ten people each session from the Hemophilia Foundation and NHLI.

Objective: To present specific scenarios based on the critical areas identified in Phase III and designed to stress the system in order to produce realistic simulations of potential outcomes. All participants in a game would in essence play themselves and represent their institutions' interests. Each gaming situation would probably achieve the following:

- Clarification of issues.
- Identification of common and conflicting viewpoints among the participants.
- Creation of new solutions.

Each game would analyze *process* and *substance*, and the results would be used for future modification of the flow chart.

Phase V - Flow Chart Modification and Preparation of Recommendations

Schedule: A one- to two-day session in June 1975 with primarily the same people participating in Phase III.

Objective: To modify the flow chart model according to the insights gained in Phase IV. Definitive activities would then be outlined and programmed at this or subsequent sessions.

OTHER POSSIBLE OUTCOMES

Some beneficial side products might evolve out of the planning process, such as educational packages that could be used in situations as disparate as high school social studies and graduate courses on health care administration. The gaming material developed here might be turned into educational units that would allow students, at whatever level, to participate vicariously in actual health decisions.

Part Two

HEMOPHILIA PLANNING MEETINGS: OCTOBER 14, 1974 AND FEBRUARY 3, 1975

The purpose of these planning meetings is to construct a theoretical model for hemophilia treatment, and to identify areas of disagreement or confusion as the subjects of two later planning sessions which will use gaming techniques. The theoretical, and to a certain extent arbitrary, nature of the model must be emphasized. While this model does represent the thinking of those present, it has been used only as a basis for departure, and is therefore certainly subject to considerable modification.

PROGRAM GOALS AND DIRECTIONS - THE TREATMENT OF HEMOPHILIA (INTERIM STATEMENT)

The central concern of this effort is to design and elaborate a system of care which will have maximal beneficial impact upon the hemophiliac with minimal disruption of the blood supply system. Attention has also been given to the phased development of this system over a ten-year period arbitrarily divided by five- and ten-year goals.

Goals

In the five-year period, the primary patient-related goal was stated as:

> "Physical freedom for the patient through adequate home care and treatment for physical deformity."

Closely connected to this goal would be the useful participation by the patient in the job market, society as a whole, and family life. As a general statement, this goal would apply to the ten-year period as well.

Objectives. The objectives of the five-year period were as follows:

1. Evaluation, improvement, and standardization of treatment, facilities, and products.

2. An epidemiologic study of patient distribution and condition. Compilation of a Registry of Patients.

3. Further research to determine prevalence, risk, and effective management of inhibitors.

4. Universal entitlement to provide adequate payment mechanisms for patient support.

5. A relevant education/information program to insure informed participation in the health care system by patients, the health care professionals, and the public. Included in this, but with slightly different emphasis and techniques, would be an education program on legislation directed at national, state, and local bodies.

6. Strengthening of patient organization, primarily through the National Hemophilia Foundation, to provide personal and group support for patients.

7. Provision of adequate amounts of blood products of suitable quality at reasonable cost.

8. Development of comprehensive diagnostic/care centers sufficient to cover 50% of the patient population evenly distributed across the country. The point here is that simply covering the East Coast (even though that may cover 50% of the patients) would not be adequate.

The creation of comprehensive care centers was seen to be not only a major goal, but also an essential or pivotal *mechanism* for achieving the other goals.

Ten-Year Goals. The stated objectives of the ten-year period were less deatiled:

1. 100% patient coverage through a regional system of comprehensive care facilities.

2. Development and availability of a safe, stable, inexpensive, synthetic product.

PROGRAM DEVELOPMENT

A potential mechanism for the achievement of the goals described above would be the creation of a network of comprehensive care centers country-wide, supported by an interconnecting network of satellites. The rationale for this approach derives from the fact that the hemophiliac requires a closely coordinated, interdisciplinary approach for treatment and support. However, the geographical dispersion of the identified patients is such that economics preclude adequate representation of needed services in all areas.

To achieve the defined goals, the following activity steps were suggested (see attached flow chart for illustration):

Activity A - Creation of a National Hemophilia Care Quality Review Board

Activity B - Data Collection and Evaluation

Activity C - Payment and Funding Rationale

Activity D - Protocol to Standardize Centers

Activity E - Evaluation of Existent Facilities

Activity F - Development of Pilot Centers

Activity G - Satellite Programs

FIGURE 2

SUMMARY FLOW CHART

Activity	Data Collection	Approximate Time: Running	Clock
1. Instruction of players a. General character of what will happen b. Roles they are to play c. Division into sub-groups	Tape	10 minutes	9:15-9:25
2. Presentation of Scenario 1	Tape	5 minutes	9:25-9:30
3. Instructions on questions to be answered and statements to be prepared	Tape	5 minutes	9:30-9:35
4. Discussion of questions and preparation of statements (in sub-groups)	Observers (one/group)	45 minutes	9:35-10:20
5. Open discussion: presentation of statements on three questions by each group; minority reports, if any; cross-group discussion	Tape	45 minutes	10:20-11:05
6. Reformulation (if desired) by sub-groups of original statements	Observers	15 minutes	11:05-11:20
7. Brief presentation of changes; limited discussion	Tape	15 minutes	11:20-11:35
LUNCH	40 minutes	11:35-12:15	
8. Presentation of Consequence A Iterate steps 3-7 allotting 30, 30, 10, and 10 minute segments	As above	80 minutes	12:15-1:35
9. Presentation of Consequence B Iterate steps 3-7 allotting 30, 30, 10, and 10 minute segments	As above	80 minutes	1:35-2:55

CLOSE UNTIL NEXT MEETING

Day 2: Same as above, but replace Scenario 1, Consequence A and Consequence B with Scenarios 4, 5, and 6.

Activity A - Creation of a National Hemophilia Care Quality Review Board

At present, no single organization with regulatory power reviews hemophilia care. Both the NHLI (DBDR) and the National Hemophilia Foundation exercise overview functions, but not in any systematic way. If a truly comprehensive care program is to be created and provided through comprehensive care centers and satellites, some knowledgeable and authoritative agency or group must be designated to plan, initiate, and monitor the system. For example, treatment standards must be set for the centers. Qualifications for center designation must be formulated. New centers must be sited in the most appropriate places. A National Quality Review Board (NQRB) could perform such functions, and the effectiveness of the total program proposed here will be directly related to the quality of such a board.

No conclusion was reached about composition or source of authority of such a board; however, several possibilities were considered. The board could exercise its function either as a *moral* or a *legal* force, or as both. Clearly, the board should be composed of recognized authorities in the field, with sufficient representation from patient and other groups to be effective. The function of the NQRB might be materially improved if it had certain legal or quasi-legal attributes, such as:

1. Legal charter from the Federal government - This might come from special legislation or through an existing organization such as PSRO.

2. Funding Control - At present, funding for the care of hemophiliacs occurs on a per patient basis from multiple sources. Assuming that National Health Insurance is enacted, single source funding becomes a possibility, and through such funding programmatic control may also be realized.

3. Product Control - Assuming that the American Blood Commission (ABC) develops as expected, and that the present dual (voluntary and commercial) blood system is unified, the production and distribution of blood products will also be controlled. The hemophilia care system, at least on the regulatory side, would be substantially strengthened, provided that the NQRB were delegated authority or review function from the American Blood Commission relating to the use of blood and blood products for hemophiliacs.

In summary, the creation of a central review agency might be essential for the successful development of any program. Its membership, responsibility, and authority must be determined.

Activity B - Data Collection and Evaluation

Despite the Booze Allen study (1972), perhaps more is *un*known than known about the hemophiliac and his treatment. Therefore, an essential first step would be the establishment of a data collection and evaluation system to study:

1. Patient distribution.
2. Social assimilation of patients.
3. Availability of treatment and other patient services.
4. Qualification and availability of appropriate health manpower.
5. Payment mechanisms.
6. Availability and quality of blood resources.

The development, control, and maintenance of the hemophilia care system obviously depends on an adequate data base. Existent data, such as it is, can be used to set standards for the preliminary efforts in developing a Centers program. However, prior to any national extension of centers, more accurate information must be collected.

Responsibility for data collection must be advantageously placed; the National Quality Review Board would be a logical site. Close collaboration between the NQRB and the ABC, however, is essential to coordinate need with flow of blood products.

Question: Should the NQRB operate as the delegated agent of the ABC for the collection of information on blood usage and need? If it did, the NQRB could gather the relevant blood data along with other data peculiar to the care of the hemophiliac.

Activity C - Payment and Funding Rationale

Currently, payment for patient services and capital funds for program development come from multiple sources. Funds from whatever source are better than no funds at all, but some *secure* base of capital funding will be necessary for the stable evolution of the centers program. Efforts must be made soon to estimate long-term development costs and to ascertain their source.

Funds for patient care represent another problem. At present they come from the patients, third party payers, and state and local governments. Since systems inevitably conform to some extent to the shape of their funding base(s), and since the current pattern of funding is unsystematic, it is not surprising that hemophilia care is unorganized.

Question: Does the present attempt to secure patient coverage actually contribute to further irrationality of the present provision of care?

The answer would seem to be yes, but the immediate alternatives are limited. However, if one assumes the imminence of National Health Insurance (NHI), the possibility of a single source of funding becomes more real. Work should be done now to determine ways in which NHI will not only cover needed patient care costs, but also do it in a way that will foster the development of a rational system. Given the model used here of comprehensive care centers, NHI payment could (1) cover actual costs, (2) allow for growth and development, and (3) be system-oriented instead of patient-oriented, i.e., direct payment would be made to certified centers on the basis of patient volume, rather than to individual patients. Responsibility for the development of this payment plan might naturally be with the NQRB.

There are a number of contingencies to this approach: (1) proposed NHI must permit this approach; (2) a center certification program must be established prior to the advent of NHI; (3) centers must be operational prior to the advent of NHI; and (4) mechanisms must be established to allow for "out-of-area payment" for patient care rendered at other than the patient's "regular center."

Activity D - Protocol to Standardize Centers

Before actual centers are developed, basic standards should be established for such things as:

1. Number of patients to be served.
2. Treatment modalities available.
3. Professional staff required.
4. Inter-relationships with other community resources (i.e., hospitals, social services, employment, etc).

Activity E - Evaluation of Existent Facilities

There are presently 9-12 facilities that could conceivably qualify as comprehensive care centers. These centers should be assessed by the standardized protocol developed in Activity D above. Those centers that meet the standards should be recognized as centers. Responsbility for such assessment should be with the NQRB.

Activity F - Development of Pilot Centers

Given Activity E, the locations and quality of treatment facilities, both formally "accredited centers" and others, will be known. It may be assumed that this coverage will not be adequate, necessitating an increase in the number and disbursement of centers. This need occasions a new problem. Although there may be some consensus about *what* a center should be, up to this point no one has ever *created* one;

hence, it would be important to operate a pilot program in which two or more centers were created from ground zero. It was suggested that while one or two of these centers might be started in areas that seemed particularly receptive to center growth, attention should also be given to an area where the need may be great, but the resources not favorable.

In addition to implementing treatment requirements, these pilot centers must give detailed attention to developing system-oriented aspects. For example, pilot centers must establish workable relationships with the ABC, or, more appropriately, with the Regional Blood Center (RBC). It is by no means clear what the optimal relationship might be, and therefore a variety of organizational arrangements should be explored.

The pilot centers should also experiment with modes of payment. Under Activity C we discussed the possibility of payment going directly to the center instead of to the patient. This pattern has advantages for system formation and maintenance, but there are undoubtedly large problems in actual operation.

Special Note on Existing Centers

Centers certified under Activity E should have a defined role in the pilot programs. The primary issue addressed in the pilot program is how to develop new centers. However, organizational problems relative to the ABC and to payment mechanisms (NHI) might most appropriately be addressed through the existing centers. It might be the special responsibility of the NQRB to establish, implement, and monitor an overall program involving *all* components of the hemophilia care system.

Activity G - Satellite Programs

The widespread population of hemophiliacs, to say nothing of economic factors, makes it impossible and undesirable to create enough centers to serve all patients within a reasonable proximity. A solution to this dilemma would be the creation of satellites for centers. The process would be analagous to the center program described in Activities D, E, and F. Essentially, a standard protocol for satellites could be developed by the NQRB. Existing facilities that might qualify as satellites should be evaluated by the protocol; those qualifying would be certified as satellites.

This process should give an accurate estimate of present capability and should orient future development. Pilot projects to develop satellites could then be initiated and evaluated. Special attention must be given to the *relationship* between the satellites and the treatment centers. Management structure, patient flow, and funding should be designed in various ways to provide alternatives and the

best possible options for various circumstances. Attention should also be given to ensuring some sort of representative geographical spread for the pilot efforts.

Responsibility for the development of the satellite program and the certification of satellites might belong to the NQRB. However, it would seem reasonable to expect that each center (new or existent) should have the responsibility for elaborating a satellite program that will be responsive to local needs. In the final analysis, the NQRB could have responsibility for reviewing and approving overall center and satellite programs.

SEQUENCING OF PLAN DEVELOPMENT

There are four major forces operative in the development of this system:

1. The need to provide adequate coverage for the hemophiliac as quickly as possible.
2. The rate of development and impact of National Health Insurance.
3. The rate of development and impact of an American Blood Commission and its regions.
4. The practical and political problems attendant to the organization and deployment of the Comprehensive Care Center and Satellite Programs.

For the system to be maximally effective, certain components and related systems must come "on line" simultaneously. Placing actual dates on these happenings is somewhat problematic, but the sequencing is relatively clear.

Impact Point I

National Quality Review Board and American Blood Commission. Prerequisite to the development of the total system as outlined is the close cooperation of the NQRB and the ABC. Therefore, the NQRB (no matter what its form) must be effectively organized and in place to interact with the ABC.

National Quality Review Board and National Health Insurance. The form of NHI and its governance is by no means determined; however, at Impact Point I, the NQRB must have a close relationship with the developers of NHI to ensure compatible development of both systems.

Impact Point II

When the ABC/RBC and NHI become operative, the comprehensive hemophilia care system must be ready to deliver because control of product and payment will be essential to development and maintenance of the hemophilia system. If no effective hemophilia system exists

when both product and payment are available, it is to be expected that both product and payment will move "outside" to the patient with no regard for any system.

ISSUES TO BE DISCUSSED

The Support of Research

During the discussions, the issue of research and its place in the overall system development was discussed with no agreement. Essentially, two positions emerged. On the one hand, it was argued that payment for research costs should be a part of the total reimbursement for center costs. This in essence would be basic, non-differentiated support, and might come as a sort of "surtax" on patient care charges. Clearly, this would provide a relatively stable support base for research.

On the other hand, it was argued that the funds for research should come in essentially the same way as at present, i.e., through NIH and foundations on a project-by-project basis. The rationale was that the present method of approval ensures quality by peer review prior to award and accountability by isolating research funds, thereby showing where they went.

The Education Program

Discussion of the education program was deferred pending further development of the system design through the gaming process. Nevertheless, several areas of need emerged:

1. Education of the public and of legislators to describe and advance the development of the system.
2. Education of health professionals to ensure informed input and participation in the system.
3. Patient education to ensure informed utilization and informed critique of the system and thereby secure system modification appropriate to patient needs.
4. Education about methods and management of hemophilia for patient and physician.

THE NEXT STEPS

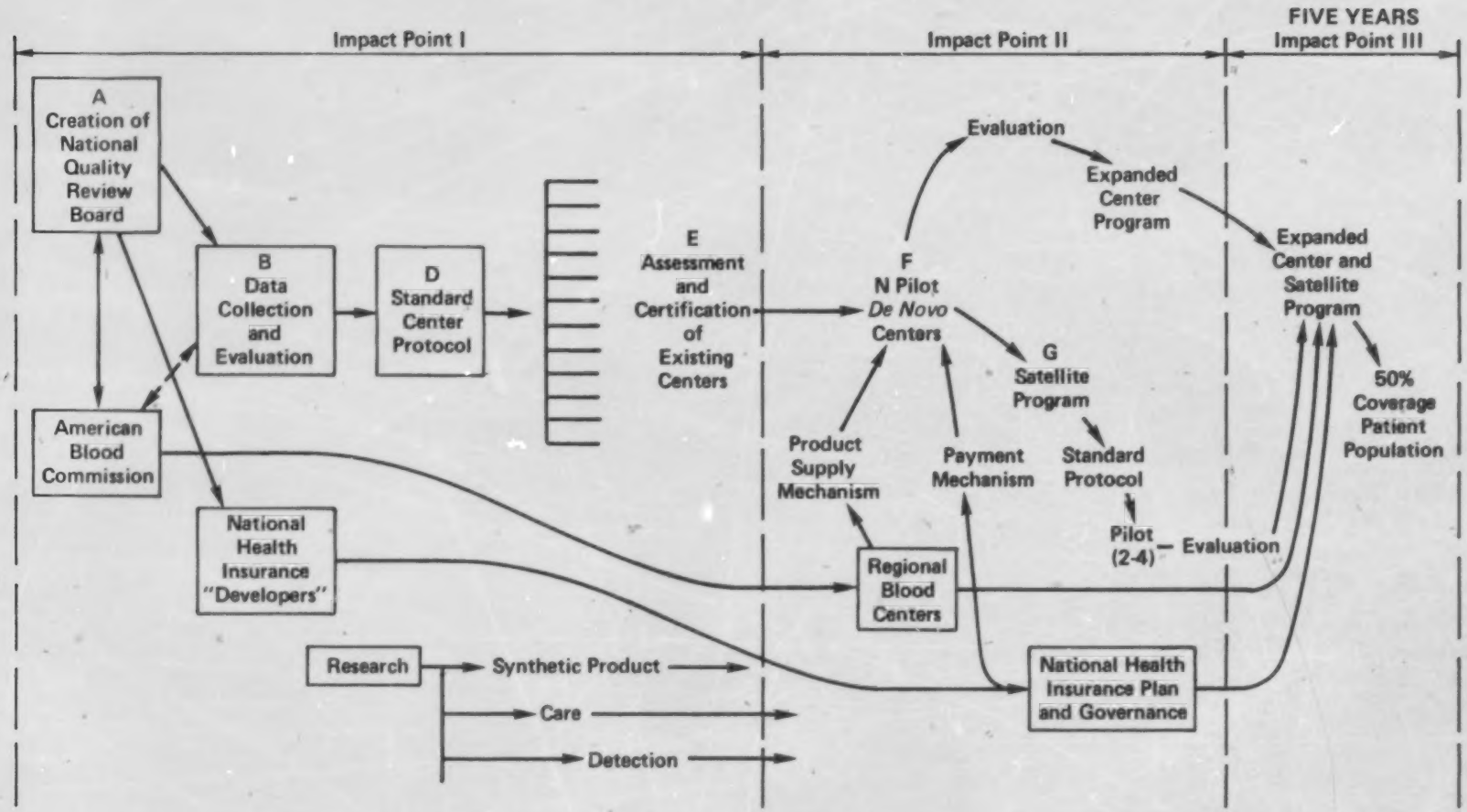
The preceeding material has described a hypothetical model of care for the hemophiliac based on the thoughts of the experts in the field. Many important areas remain to be explored, and that exploration will be the central concern of the gaming process. The following specific issues might be addressed in these games:

1. The Impact of National Health Insurance.

2. Mandated Home Care. The model described posits a central focal point in centers and satellites. Funds would flow through the centers, and patients would relate essentially to these centers. The logic and structure of this program would be severely tested if the impetus moved from the center to the home. If funding shifted from the center to the patient, who would negotiate for his care? Question: Could the system survive under these circumstances, and if so, what would the cost be?

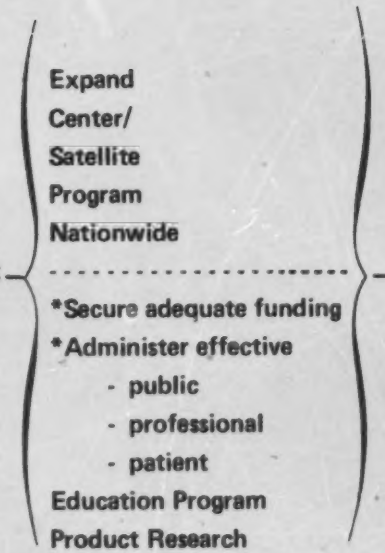
3. Other Issues. Three other issues might be considered, though possibly not incorporated into complete individual games:

- Composition and Role (Authority) of the NQRB.
- Support or Resources for Research.
- Appropriate Role of Education.


Impact Point I
Impact Point II
FIVE YEARS
Impact Point III
A
Creation of National Quality Review Board
B
Data Collection and Evaluation
D
Standard Center Protocol
E
Assessment and Certification of Existing Centers
F
N Pilot
De Novo
Centers
Evaluation
Expanded Center Program
Expanded Center and Satellite Program
G
Satellite Program
50% Coverage Patient Population
American Blood Commission
National Health Insurance "Developers"
Product Supply Mechanism
Payment Mechanism
Standard Protocol
Pilot (2-4)
Evaluation
Regional Blood Centers
Research
Synthetic Product
Care
Detection
National Health Insurance Plan and Governance

FIVE YEAR GOALS

1. Freedom for Patient
   (a) Home Care
   (b) Repaired physical deformity
2. Evaluation and standardization treatment, facility and product
3. Epidemiological study of patient distribution and condition
4. Management of inhibitors
5. Universal entitlement
6. Strengthened patient organization
7. Adequate (quantity and quality) blood product
8. Development of center/satellite program for 50% of patient population



TEN YEAR

1. 100% coverage Center/ Satellite Program
2. Safe, stable and cheap product (oral or intramuscular)
3. Possible prevention

Part Three

OUTLINE OF SESSION I

I. Instruction of Players

- A. General Character of What Is to Happen
- B. Roles the Players Are to Assume
- C. Division of Participants into Subgroups

II. Presentation of Scenario 1

III. Instructions on Questions to be Addressed and Statements to be Prepared

- A. Catastrophic Disease National Health Insurance Act
  - 1. Group Statements by Participants
    - a. Product Sector
    - b. Care Sector
    - c. Payment Sector
    - d. Education and Research Sector
  - 2. Floor Discussion:

    Care Sector
    Product Sector
    Payment Sector
    Education and Research Sector
  - 3. Reformulation of Opening Statements
    - a. Product Sector
    - b. Care Sector
    - c. Payment Sector
    - d. Education and Research Sector
- B. Presentation of Consequence A:
  13,000 Additional Hemophiliacs Entering the System
  - 1. Group Statements by Participants
    - a. Education and Research Sector
    - b. Payment Sector
    - c. Product Sector
    - d. Care Sector

2. Floor Discussion:

Education and Research Sector
Product Sector
Care Sector

C. Presentation of Consequence B:
$5,000 Ceiling on Hemophilia Care Per Individual

- Group Statements by Participants

a. Research and Education Sector
b. Payment Sector
c. Care Sector
d. Product Sector

OUTLINE OF SESSION II

I. Instruction of Players

II. Presentation of Scenario 2

A. Establishment of 15 Regional Centers

1. Group Statements by Participants

a. Care Sector
b. Education and Research Sector
c. Payment Sector
d. Product Sector

2. Floor Discussion

B. Presentation of Consequence A:
Five Centers Named to Carry on Hemophilia Research

- Group Statements by Participants

a. Product Sector
b. Payment Sector
c. Care Sector
d. Education and Research Sector

C. Presentation of Consequence B:
National Hemophilia Centers to Become Depots for Blood Products

1. Group Statements by Participants

a. Care Sector
b. Education and Research Sector
c. Payment Sector
d. Product Sector

2. Floor Discussion

HEMOPHILIA GAMES: SCENARIOS AND RESPONSES

SESSION I
March 10, 1975

I. INSTRUCTION OF PLAYERS

A. General Character of What Is to Happen

Over the course of the next few hours, and then continuing next time we meet, what we are going to do is to act as if some events that seem *likely future developments* have actually taken place. Some of these may seem extremely fortuitous events with positive consequences for those concerned with hemophilia care, while others will seem less promising if not highly threatening to your interests and concerns! We would assume that all will eventually prove to have a mixed character upon further exploration.

Our concern here is as was expressed during our last several meetings: to develop a better understanding of the impacts that such potential events would have on the hemophilia system as it exists and as it is likely to develop in the next decade.

To try to tease out some of these impacts, then, we will simulate the initiation of the event by telling you what has happened, and then ask you to work in subgroups to develop responses to the event that will help unearth some of the less obvious dimensions of the system and its stress points. Thus, your thoughtful and thorough responses to the event should help reveal where we may need to work to make those things you desire come about, and where work may be needed to prevent other consequences that emerge in the simulation from coming into being in reality.

B. Roles the Players Are to Assume

In a few minutes, we will present the first event and explain the terms in which we would like you to respond to it. But first, let me explain something about the role we hope you will play.

All of you gathered here represent important contributors to the actual planning of hemophilia care in this country because of the special expertise you have demonstrated in your work and the eminence you've gained from it. At the same time, you represent for purposes of gaining better understanding of the shape and character of the hemophilia care

system, *a type of interest or constituency*. For example, Dr. Aledort is the director of a care center at Mount Sinai, and as such has a general understanding of the concerns and needs of physicians who treat hemophiliacs and who administer treatment centers. Dr. van Eys not only does his own research, but has a general understanding, as a researcher, of the needs and problems and concerns of other researchers in other places, albeit they may work on a smaller scale or in less well-equipped surroundings. What we are asking you to do is to try to respond to the events and the questions in those more general roles, rather than in your specific roles. That is, when we ask, "How will this affect you?" we are asking "How will this affect people in your sector?".

To answer in these general roles will sometimes be complex and require that you say something such as, "Those in small ill-equipped centers will do X and those in major centers will do Y." That type of distinction, however, is critical for our understanding of the impacts of the events, and you are the people best equipped to deal with such questions and nuances.

C. Division of Participants into Subgroups

To get us started in the first game, you will be divided into subgroups, each representing one of the major sectors of the hemophilia care system. Some of the activities of the day will take place simultaneously by each group, while others will involve you in one large group discussion. Therefore, we are seating each group at a separate table spaced far enough apart to permit private discussions, but close enough together to allow subsequent full discussion.

The four subgroups represent concerns with PRODUCT, PAYMENT, CARE, and RESEARCH AND EDUCATION. Within each will be people with that general concern and with special expertise in that area; but, as you will notice, the group may not be homogeneous in the *nature* of their interests. For example, the PRODUCT group contains representatives of the private and the public spheres who have both convergent and divergent interests in product development and dissemination.

II. PRESENTATION OF SCENARIO 1

We are ready, then, for the first "News Bulletin." The event you are about to hear is to be considered a "fait accompli." If you don't like it, it's too late to do anything about it; if you do like it, it's too late for anyone else to alter it.

HEW NEWS

U.S. DEPARTMENT OF HEALTH, EDUCATION, AND WELFARE

PRESS RELEASE
Monday, March 10, 1975

National Institutes of Health
William E. Sanders (301) 496-5343

WASHINGTON, D.C.--Congress today overwhelmingly approved an all-inclusive Catastrophic Disease National Health Insurance Act. In a carefully orchestrated move by Senate and House leaders, the compromise bill which had gone to a conference committee to resolve differences was introduced and passed by the two Houses in the same day. Leaders of both Houses stated that the large congressional majority assures the President will sign the new compromise bill into law, or face a certain veto over-ride.

Never again, said the bill's supporters, will Americans be made to suffer financially from debilitating disease. All U.S. citizens and residents will be covered under the Catastrophic Disease Health Insurance program.

NEW PAYROLL TAX

The new program will be financed by a 3.5% tax on employers' payrolls, a 1% tax on employees, and 2.5% on the self-employed up to $20,000, with the remainder coming from general revenues. All monies collected are to be paid into the National Catastrophic Health Insurance Fund. The Fund will be administered by the newly created Catastrophic Disease Health Insurance Board established under the Department of Health, Education, and Welfare.

The Board will be advised by the new Catastrophic Disease Health Insurance Council composed of a representative group of consumers, providers, health organization personnel, and other interested parties.

MEDICARE AND MEDICAID STAY

Both the Medicare and Medicaid programs which provide preventive and catastrophic benefits will remain intact, except that the same benefits established under the Catastrophic Disease National Health Insurance Act will be extended to include these programs. It is likely that Federal and/or State representatives involved in administering these programs will sit on the Catastrophic Health Insurance Council.

BENEFITS

The Act provides that the first $500 spent annually on medical care must be borne by the family or individual suffering from the disease. Thereafter, the government will pay for necessary disease-related treatment--all physician care, hospital days, outpatient care, nursing home stays, supportive services, rehabilitation needs, psychiatric care, inpatient and outpatient drugs including blood and blood products, and prosthetic devices. The program does not cover health needs unrelated to the disease.

REIMBURSEMENT

Reimbursement will be similar to the current Medicare program. The physician bills the patient who files a claim for the reasonable cost allowed by Medicare. Under the Catastrophic Disease program, reasonable cost can be determined by the Catastrophic Disease Health Insurance Board in advisement with the Catastrophic Disease Health Insurance Council.

$25 MILLION FOR PLANNING

The actual start up date for the Catastrophic Disease National Health Insurance program will be January 1, 1977. Until then, the Act calls for the expenditure of $25 million over a two-year period to plan in detail how the program will work. It is hoped that with adequate planning the program will run smoothly once it is implemented.

III. INSTRUCTIONS ON QUESTIONS TO BE ADDRESSED AND STATEMENTS TO BE PREPARED

Keeping in mind the role you are playing, there are three questions you need to consider with respect to the news you have just heard. At the end of 45 minutes, we need from each group a detailed statement about these three questions to be presented to the group as a whole for its discussion and debate. Following that there will be a short period for reformulations in light of what others have said; and, finally, a brief discussion of these reformulations will take place before we move to the next event.

The questions all have reference to the *first year following the event* and are as follows:

1. HOW WILL THIS EVENT AFFECT YOU?

2. WHAT WILL YOU DO OR TRY TO DO BECAUSE OF IT? (i.e., How will you alter your behavior, operations, etc.?)

3. WHAT SHOULD OTHERS IN THE SYSTEM DO ABOUT IT? (Product, Care, Payment, and Research and Education interests)

Please try to be as specific as you can in responding to these questions. We hope you will work to define the common elements of your responses through discussion in each group, but minority reports on any dimensions are perfectly appropriate where there is dissent.

Let me also note that we are placing an observer at each table to help collect more detailed reports than you will be able to make in the open discussion. These people will not be interfering in the discussions.

A. CATASTROPHIC DISEASE NATIONAL HEALTH INSURANCE ACT

1. Group Statements by Participants

a. Product Sector

The Product Sector considered the impact of this new legislation from two viewpoints, the profit and nonprofit sectors. The profit sector, mainly pharmaceutical companies, will see it as an opportunity to increase production. A 30-50% expansion is realistic in terms of plasmapheresis, especially if untapped sources of plasma donors are used. One such untapped plasmapheresis source is the university population. The manufacturers will be able to make more antihemophilic factor, sell more, and make more money. We will meet the demand.

For nonprofit blood bankers, our initial response will be relief from a large headache--hemophiliacs' bills will be paid for the first time. Problems: (1) There will be an increase in demand. (2) Patients may want highly purified product which is very expensive to produce. To obtain highly purified product, it is necessary to throw away a large amount of the plasma fraction. Currently, about three quarters of the fraction we start with is thrown away.

Both the profit and nonprofit sectors will have financial and logistic problems supplying this increased demand. Both will have to expand to provide the best product.

It will also be necessary to educate physicians to use blood products more efficiently. If cryoprecipitate is the most efficient, then they will just have to use cryoprecipitate and not the fanciest product available.

It also must be stressed that hemophiliacs are one of a number of groups who need blood. They will have to share this resource. Hemophiliacs ought to make an effort to expand the nation's blood resource.

b. Care Sector

This new legislation will result in a massive demand for care and could lead to the breakdown of the care system. Most comprehensive care is sparse--sparse staff and facilities. Now most programs give care only on a part-time basis, perhaps once a week. We will need a much larger program, operating on a daily basis. Daily operation will necessitate centralization. Currently, there is no centralization or coordination among programs. Even in large cities, there are no coordinated programs.

A significant concern is that the center program may not be established. Currently, individual hematologists prefer not to treat hemophiliacs because they do not get paid adequately for their services. But when the individual hematologist discovers that now he can make a living from treating hemophiliacs, he may be reluctant to give up these patients. To counteract this, there needs to be something like the House of Representative's bill H.R.I, which proposes establishing a medical program concerned with the type of care that is delivered, who is a qualified provider, and what is a reasonable charge for services. A Hemophilia Commission empowered under such legislation or created by executive order could endorse the center system.

The Care Sector would fight hard for a Hemophilia Commission. The Commission could endorse a group provider for hemophilia care and de-emphasize the single physician. Hopefully, it would also de-emphasize prophylaxis at present time because of lack of data and product. Because hemophilia care is tied directly to this country's blood supply system, this Hemophilia Commission would have to work with the American Blood Commission (ABC). The Hemophilia Commission would work with blood bankers to set standards of care immediately following the enactment of this catastrophic health insurance legislation.

Even if the center concept is endorsed, there may not be enough material to treat hemophiliacs. The price will skyrocket. Patients may demand prophylaxis--a real problem. The blood service complex would have to work more effectively. The only way to meet the expected increase in demand is to regionalize the entire blood supply system and have it work with the Hemophilia Commission.

Care models will be needed to demonstrate the best delivery of care and treatment. These models could become standards of care. Then it becomes a matter of educating both the lay public and the professional. We have to inform the public and train the professional about the comprehensive approach to hemophilia treatment.

c. Payment Sector

The lead time in this legislation allows enough planning time to write well thought out rules and regulations. The rules must answer these questions:

1. Who will be entitled to coverage?
2. What diseases will be covered?
3. What is disease-specific treatment?
4. What are the benefits?
5. How much will the Federal government pay?
6. Will only qualified providers get coverage?

Furthermore, these rules and regulations should define the institution and the physicians. Those who write these legislative guidelines will also have to examine the effect of payment on blood product. Demand for blood products will rise and the available dollar will be chasing the available product. How to control price will need to be clearly examined. The ABC may be able to control price when plasma is available able on a systematic basis.

d. Education and Research Sector

This legislation will have a tremendous effect on hemophilia. The first goal of the National Hemophilia Foundation (NHF) would be to get knowledge of the bill out to all treatment centers. Treatment center personnel will be greatly concerned that effective treatment might decline. The hemophiliac may become apathetic about what happens to the disease. He may take an "I am taken care of" attitude. Many will go on prophylaxis.

Education will be altered. The National Hemophilia Foundation and the ABC will have to increase product knowledge and promote the optimal use of component therapy. The NHF, through its Medical and Scientific Advisory Committee, would encourage the Council established by this legislation to promote blood research. The NHF will also take steps to assure that hemophilia is legally defined and that hemophiliacs have entitlement to care through comprehensive centers.

How will this legislation affect research? Researchers and providers have to prevent the standard of care from deteriorating. More hemophiliacs will be entering the system. Increased numbers will mean that for a time the level of care will decrease and be lower than it now is. It may seem like a retreat. And this lower success rate may affect research. A pocket of money must be reserved for research into blood and blood products. Also, we need enough data so we can make good use of product. There must be a concerted effort for research on all blood products; synthetic AHF research would be stimulated too. The pattern of research would be first research into treatment, then research to find a cure, and then prevention.

2. Floor Discussion

Care Sector: We must answer the question on prophylaxis. We will not have the definitive answer for 10 years, but we need this answer. HEW would turn to the National Heart and Lung Institute and say, "What is the best care?" To answer this question, we need money

and an ability to mobilize a constituency to say that certain data are critically needed if hemophilia care is going to be efficacious and cost-efficient.

Product Sector: How would you approach the NHLI Blood diseases and Resources Advisory Committee when we are in economic slump and when clinical investigations are a low priority? What kind of support would you need to alter this mindset? How would you do it?

Payment Sector: How do we begin to get an answer? You can turn to the government agencies if not for efficacy, then for standards of care, and you can begin to look at this. It would be useful if tentative guidelines were established--a mechanism needs to be established.

Education and Research Sector: One practical step that can be taken now would be a workshop--like the recent albumin workshop. The workshop could summarize the state-of-the-art, issue tentative guidelines, and identify further data needs and capabilities relative to prophylaxis.

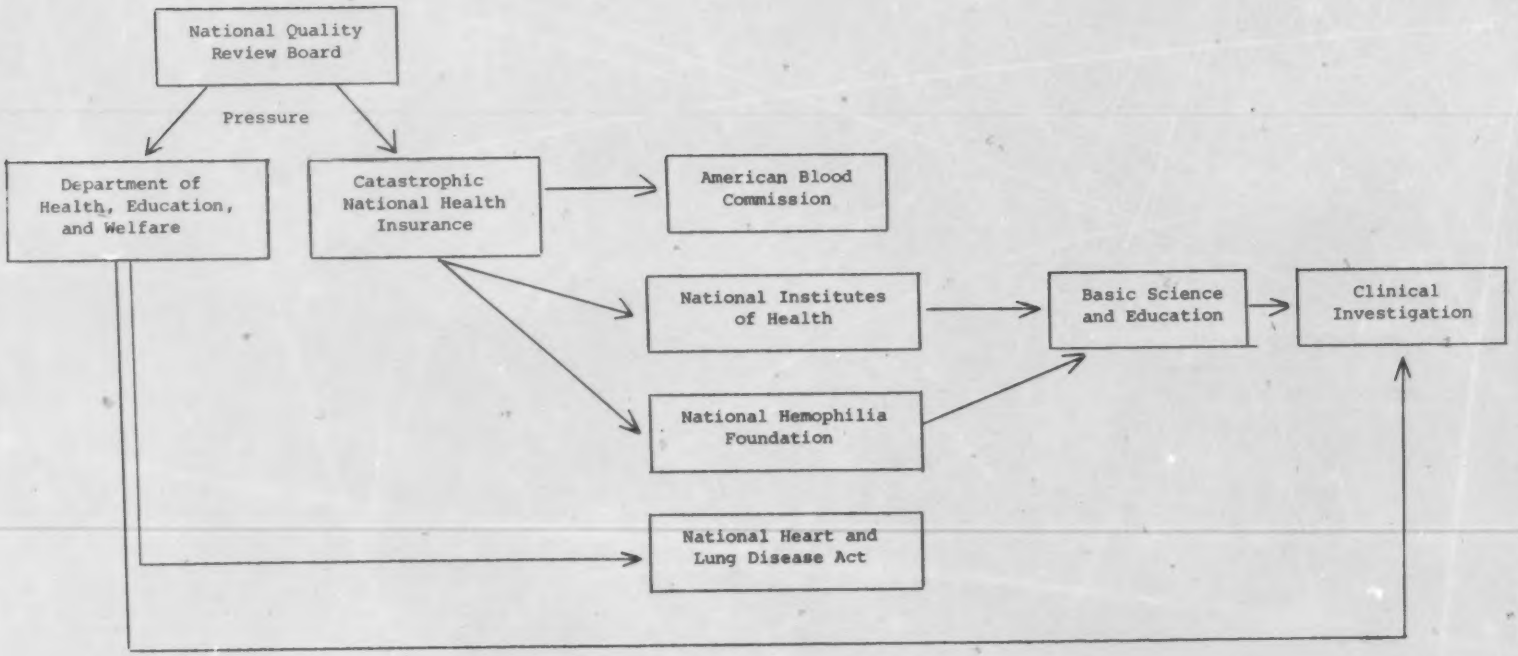
3. Reformulation of Opening Statements

a. Product Sector

The pharmaceutical firms claim that the potential supply of plasma is unlimited, provided money is no object. The limiting factor is plant capacity. Will the government loan money? The cost of Factor VIII is moderated by other types of blood derivatives produced from that same plasma. As the demand for Factor VIII rises and demand for albumin drops, it is going to cost more for Factor VIII. The data on this are very limited. Really, we have data for only one year, 1971. The rest is anectodal; we are guessing. Our group endorses: (1) the need to start the machinery for data, and (2) the ABC should act as the focal point for collecting this data nationwide.

b. Care Sector

We have drawn a diagram to explain the forces at play in this situation. The National Hemophilia Commission could apply pressure to HEW, influencing standards of care, etc. The HEW will have input into the implementation of National Health Insurance; also the Commission would influence the implementation of the law. HEW also has a great deal to say on how much and what kind of clinical investigation is undertaken.


National Quality Review Board
Pressure
Department of Health, Education, and Welfare
Catastrophic National Health Insurance
American Blood Commission
National Institutes of Health
Basic Science and Education
Clinical Investigation
National Hemophilia Foundation
National Heart and Lung Disease Act

The repercussions of this National Catastrophic Disease Health Insurance Act would be felt by the American Blood Commission, the National Institutes of Health, and the National Hemophilia Foundation. The National Institutes of Health and the National Hemophilia Foundation together would affect basic science research and education. Basic science research and education, in turn, influences the state-of-the-art of clinical investigation which is funded by HEW.

c. Payment Sector

We will address ourselves to product and its cost. It is not likely that the government will move to alter the method of payment in the free market. According to industry, if price is not controlled, there will be unlimited product. To counteract skyrocketing prices, we have developed a strategy to control price. We endorse establishing one or more Federally supported fractionation centers as a benchmark for cost. It (they) could regulate the market and thereby control the price.

This Federally supported fractionation center could become the recipient of pooled plasma from regional blood centers via the ABC. Then, these products priced at cost will be the competition. Regulating price in this country will result in driving the pharmaceutical companies to sell the concentrate abroad in pursuit of profits. By developing a competitive nonprofit fractional center, we indirectly control the profit sector without forcing blood products abroad.

d. Education and Research Sector

The NHF will play the role of a consumer protection agency for all blood diseases. Its role will be to define to patients what good care is; the NHF will provide consumer education to patients.

B. PRESENTATION OF CONSEQUENCE A

You spent the morning thinking about and talking about the effects that an event would have on you and the ways it might alter your behavior. At the same time, though you couldn't see them and they didn't make a presentation here, others were viewing what happened during this first year, and we have another "News Bulletin" concerning that:

13,000 ADDITIONAL HEMOPHILIACS ENTERING THE SYSTEM

"As a result of the new coverage offered hemophiliacs in the Catastrophic Disease National Health Insurance Act, a large number of hemophiliacs previously unknown to the hemophilia

system have appeared. In a period of about two years, some 12 or 13 thousand new patients came for treatment. These patients were divided between mild sufferers who were seeking more adequate and frequent treatment and those more severe cases suffering from the chronic affects of hemophilia. This latter group was older, had more severe disabilities, and were poorer, less skilled, rural persons who are now seeking adequate health care. These persons stress the system in very particular ways since they need a good deal of orthopedic surgery, dental care, and psychological and vocational rehabilitation in order to take their place in society. All of the new patients, regardless of the severity of disease, require full diagnostic work-ups and the usual tests that are given to the hemophiliacs."

You will find the current hemophiliac population described in the following tables.

"13,000 MORE HEMOPHILIACS SEEK CARE"

	Past Hemophilia Population			Current Hemophilia Population		
Type of Patients	Total	Factor VIII	Factor IX	Total	Factor VIII	Factor IX
Severe	14,421	12,117	2,304	19,867	16,479	3,388
Moderate	11,078	8,180	2,898	18,632	14,320	4,312
Total	25,499	20,297	5,202	38,499	30,799	7,700
	Past Distribution of Type by Severity			Current Distribution of Type by Severity		
Severe	56.6	59.7	44.3	51.6	53.5	44.0
Moderate	43.4	40.3	55.7	48.4	46.5	56.0
Total	100.0%	100.0%	100.0%	100.0%	100.0%	100.0%

53

SOCIO-ECONOMIC CHARACTERISTICS
OF HEMOPHILIACS

Living Arrangements	Percent
With Parents	72
With Spouse	22
With Other Relatives	3
Alone	3
Other	0
Father Living	
At Home	75
Not at Home	25
Annual Income	
Less than $3,000	6
$3,000 - $6,999	21
$7,000 - $9,999	20
$10,000 - $14,999	27
$15,000 or More	25

GEOGRAPHIC DISTRIBUTION OF
HEMOPHILIACS AND TOTAL MALE
POPULATION OF THE U.S.

Geographic Region	Hemophiliacs (%)	Males in U.S. (%)
New England	6.8	5.8
Mid Atlantic	25.9	18.0
East North Central	15.6	19.8
West North Central	9.8	8.0
South Atlantic	10.1	15.1
East South Central	3.8	6.3
West South Central	8.5	9.5
Mountain	3.7	4.1
Pacific	15.7	13.2
Total U.S.	100.0	100.0

AGE DISTRIBUTION OF HEMOPHILIACS IN THE U.S.

Age Group	1971 Percent	1971 Cumulative Percent	1977 Percent	1977 Cumulative Percent
Under 3	8.9	8.9	8.9	8.3
3 - 4	15.3	24.2	10.3	19.2
5 - 9	19.8	44.0	12.8	32.0
10 - 14	25.1	69.1	17.1	49.1
15 - 19	12.7	81.8	11.2	60.3
20 - 24	6.5	88.3	10.9	71.2
25 - 34	7.3	95.6	9.5	80.7
35 - 44	21.	97.9	8.3	89.0
45 - 54	1.4	99.1	5.6	94.6
55 - 59	0.3	99.4	3.7	98.3
60 - 64	0.3	99.7	0.7	99.0
65 - 74	0.2	99.9	0.6	99.6
75 +	0.1	100.0	0.4	100.0
Total	100.0		100.0	

Median Age: 11.5

Median Age: 17

This surely will also have an effect on you and require some modification of your activities. What we would now like to do is to ask you to repeat the steps you engaged in earlier. This time, however, the time available to you for each step will be considerably shorter.

1. Group Statements by Participants

a. Education and Research Sector

These 13,000 hemophiliacs will stress the system more because of increased numbers and their general health neglect. These patients will require more rehabilitation than care; as a result, centers may have to set some priorities. Rehabilitation may be a problem, since it is ancillary care. Ancillary care may not be a reimbursable item.

More hemophiliacs will accentuate the need for research. But to bring this new group up to the established median level of rehabilitation will be difficult. This may hinder research because progress in the patients' condition will not be apparent.

b. Payment Sector

The payment group feels that these additional hemophiliacs will increase the amount of money which the government will have to spend on this disease. It is analogous to what happened to renal disease and Title XIX. Congress was furious, but it was only able to raise the deductible. The demand for ancillary services will increase. The government may have to increase the amount of payroll taxes, but once it has extended coverage, it cannot reverse itself. The National Hemophilia Foundation should try to obtain a large planning grant to determine how many hemophiliacs there will be in the event that this kind of national health insurance legislation is passed.

c. Product Sector

For those of us in the profit sector, more hemophiliacs will improve our profits picture; that is, if the government will finance new plants using Federal money. This infusion of money will stimulate us to use new technology to make cheaper, better products. Then we will persuade the private sector to use these new products on patients.

What will keep our prices down? The threat of stringent price control will be our incentive. If price controls are put into effect, then we, as industry, will say we are closing down.

Congress might then react with an embargo unless the pharmaceutical industry meets the needs of 90% of this country's hemophiliacs before it ships any of its blood products abroad. And Congress will act if Americans are bleeding to death.

d. Care Sector

In our group, the assumptions that we made were the following: (1) National Health Insurance reimburses groups, not individuals; (2) programs have expanded to include all those now identified; and (3) there are good data on these new 13,000 hemophiliacs. Hemophilia is now less a problem of prevention than a problem of acute care--in essence back to where we started. This group of hemophiliacs are poorer and need more acute care. We will expand the psychosocial, vocational, and educational services, and surgical and dental rehabilitative care in phases. Surgeons will do more and more reconstructive surgery. These newly identified hemophiliacs probably have poor dental health and will require good, expensive dental care.

We think the satellite system will have to handle the brunt of this new demand for services. In the face of this new influx, we will need a good transition program capable of changing from providing care to urban patients to providing care to rural patients. Those patients who need the best care will have to go to large centers, after which they can return to their communities.

Having a satellite system will require a "traveling show"--diagnostic services, therapeutic care, and triage. The ideal way to monitor the progress of satellite care would be a television hookup. We will also need funds for special clinical training. The question is, how are we going to get funds when all the money is in research? Somehow we will have to go to the satellite center and educate the professional and his patient about hemophilia care, but funding may be a problem.

2. Floor Discussion

Education and Research Sector: But what about new technology? The technology exists for inserting relevant portions of the X-chromosome which would alleviate the entire problem. There would be no new cases of hemophilia. Estimating when this technology is feasible is difficult; it could happen in five years, depending on the social forces.

Product Sector: If we are talking about pure technology without human subjects, fine. But turning mini-research into macro-production? We'll need high risk dollars. It will still take five years to find out how to make synthetic AHF in large quantities, let alone

do genetic engineering. We do not know a great many things. Will synthetic AHF increase antibodies? We don't know. Currently, this country collects 2-1/2 million liters of plasma and we could use 50,000 more people in plasmapheresis.

The major source of Factor VIII will continue to be plasmapheresis. It is not likely that we are going to have a sufficient collection system for whole blood. Volunteerism is a major part of the National Blood Policy. Once we have to pay for plasma, this may erode and destroy the volunteer principle. If we become totally dependent on paid plasmapheresis, we may discourage volunteers. But also, what will happen if it has a negative feedback? Then, this country will be really short of Factor VIII. We may really be in trouble.

How much Factor VIII does the average hemophiliac use in a year for prophylaxis care, home care, and surgery?

Care Sector: For surgery about 100,000 units of Factor VIII is used. Also, you need a surgeon with the skill to do it. Surgeons are doing more surgery; some are experimenting with knee replacement. We need to know the floor of care.

For acute, episodic care, about 100 units of cryoprecipitate are needed, but many people go beyond this. There may be an increase in product use, caused simply by having secure payment.

C. PRESENTATION OF CONSEQUENCE B

During the full time you have been meeting and discussing, the National Health Insurance Act and the additional 13,000 hemophiliacs, yet another group has been viewing what has been going on, and we now have the last event for you to consider today. As soon as it's been read, please again prepare statements.

$5,000 CEILING ON HEMOPHILIA CARE PER INDIVIDUAL

"In the third year of the existence of the National Health Insurance Bill, after the emergence of 13,000 new hemophiliacs in the first two years of the program, Congress was struck by an appalling rise in the cost of the program. The Congress has decided to put a ceiling on the coverage given to hemophiliacs. Rather than changing the mechanism of payment or controlling the quality of treatment, they have decided that they will continue to pay either to the patient or to the patient's doctor, but that there will be a fixed upper limit of $5,000 to be given to each individual patient in any given year."

Group Statements by Participants

a. Education and Research Sector

For us in the National Hemophilia Foundation, this ceiling will mean we will have to begin again to articulate our own needs and the needs of other blood diseases. We will have to increase the funds available for research. The major emphasis will be on research--it is the only way out of this predicament.

b. Payment Sector

This ceiling is a probable likelihood. Under Medicare, nursing home care was cut back to 60 days and there was a legitimate short fall. In Congress, all Great Society Programs are being cut back and yet this current Congress is a populist Congress. In relation to the game, a way around the ceiling might be cost sharing among the states.

c. Care Sector

There was great concern that there will be a regression in treatment and a return to the episodic modality. Orthopedic care will only be on an acute basis. Psychosocial services will revert to helping hemophiliacs learn to live with their disease. It will be a more passive phase. The center system will be strengthened. The clinical team will deal more with family planning and eugenics. We lost sight of this preventive aspect earlier in the game. We would urge research in carrier detection too. In addition, there will be more pressure toward synthetic AHF research and efficacy of method in treatment.

d. Product Sector

We realized that with this ceiling we overbuilt from a base that was too large: 38,000 hemophiliacs averaging 100 bags of cryoprecipitate at $10-$12 a bag equals $40 million. Starting with this capability means that half of all the blood collected needs to be fractionated. Using the current plasma collection of 2-1/2 million liters having a 20% salvage rate results in 250 million units total. The cost is $50 million with another $90 million for the rest of all care. Using the $50 million figure, we realize now we expanded when really we did not need to. It is another instance of the well known fact that any time money becomes available, care becomes excessive.

SESSION II
April 24, 1975

I. INSTRUCTION OF PLAYERS

Today's events are a continuation of the events that occurred in the game you participated in last month. For those of you who were not here then, you represent yourself and your type of interest or constituency.

To bring you up to date and to remind everyone of what has occurred before, I will read a summary of events that have happened to medical benefits for hemophilia. As you know, on March 10, 1975, Congress passed the all inclusive Catastrophic Disease National Health Insurance Act. The Act provided for full coverage of hemophilia and all hemophilia-related health needs once the patient or his family paid for the first $500 of care. The program financed by a tax on employer's payrolls and a smaller tax on the self-employed and employees, with the remainder coming from general revenues paid into a Trust Fund. It is administrated through the Department of Health, Education, and Welfare by the Catastrophic Disease Health Insurance Board, which is advised by the Catastrophic Disease Health Insurance Council composed of a representative group of consumers, providers, health organization personnel, and other interested parties. The Act was put into effect in January 1977, following a two-year planning period.

Once the program began, many more hemophiliacs sought care than had been expected. In fact, 13,000 hemophilic patients previously unknown to the system came for treatment. Half of these patients were moderate hemophiliacs who wanted more adequate and frequent treatment. The other half were severe hemophiliacs suffering the chronic effects of hemophilia. These severe hemophiliacs as a group were older, poorer, less skilled, and mainly rural people who had severe disabilities and were seeking adequate treatment for the first time.

Both the moderate and the severe hemophiliac stressed the hemophilia care system very directly. The older, severe group required a great deal of orthopedic surgery, dental care, and psychological and vocational rehabilitation. The moderate patients, like the severe patients, required full diagnostic work-ups and, in the beginning, considerable monitoring of their disease.

The influx of these 13,000 additional hemophiliacs into the system was felt by Congress during the third year of the Catastrophic Disease Health Insurance Program. Congress became alarmed over the amount of

money that was going to pay for hemophilia treatment in proportion to the rest of its health care dollar. Rather than changing the payment mechanism or controlling the quality of treatment, Congress decided that the government would continue to pay either to the patient or the patient's doctor a fixed upper limit of $5,000 per hemophilic patient in any given year.

II. PRESENTATION OF SCENARIO 2

Now, with this in mind, I will read the first "News Bulletin."

15 HEMOPHILIA CENTERS TO BE ESTABLISHED

"Responding to pressure to improve care within the $5,000 limit set for each hemophiliac, Congress has authorized the Department of Health, Education, and Welfare to establish 15 comprehensive care centers across the United States. Each center will offer comprehensive care, have a complete diagnosis unit able to do both diagnostic tests and genetic testing, and will engage in research, education, and training.

"Forty-five million dollars has been appropriated by Congress to establish the 15 centers. Each center will cost approximately $3 million to build. Operating expenses will be funded in part by government money--the first $5,000 of care each hemophiliac receives under Catastrophic National Health Insurance. Ten percent of the income obtained by each center must be spent on research. Each center is free to decide how much of its budget it wishes to allocate for education and training.

"Attendance at these centers will be voluntary; neither the patient nor the doctor will have to use the services provided at these centers. The doctor-patient relationship, if it is satisfactory to both parties, would not be disturbed.

"Each center will have a patient population of approximately 2,500 hemophiliacs. The 2,500 patient population figure is based on the known number of hemophiliacs seeking care: 38,500. Thus, if 2,500 patients all require the government allotted benefit of $5,000 per hemophiliac, the center's total operating budget would be $12.5 million. For all 15 centers, the budget would be approximately $187.5 million.

"The number and location of hemophilia centers in a region were determined on the basis of the known geographical distribution of hemophiliacs. Within certain limits, individual hemophiliacs will be able to attend the facility nearest to them. The 15 cities chosen are Seattle, Washington; Los Angeles, California; Denver,

Colorado; Minneapolis, Minnesota; Kansas City, Kansas; Houston, Texas; Chicago, Illinois; Ann Arbor, Michigan; Nashville, Tennessee; Rochester, New York; Pittsburgh, Pennsylvania; Chapel Hill, North Carolina; Atlanta, Georgia; New York City, New York; and Boston, Massachusetts."

A. ESTABLISHMENT OF 15 REGIONAL CENTERS

1. Group Statements by Participants

a. Care Sector

We have problems because, supposedly, we have $15.5 million for each of these hemophilia centers, but we do not really have this money because the patient has the ultimate decision on where to spend his money. This is a great challenge: How can we build these new facilities when these facilities may never be utilized? The patient may choose not to use our services, then where are we? What is required is an ongoing operating budget to allow the center to operate before it has a full complement of patients.

In order to create an operational budget, we recommend initiating a requirement for a yearly comprehensive examination for all hemophiliacs to be given only at the center. This yearly examination would become the qualification for support by the center for that year or this examination report would be sent to a private physician. The private physician, once he received this report, could treat this hemophilic patient for a year.

Considering the staff and the laboratory facilities that are needed for a thorough evaluation, $500 is not an unreasonable fee. This fee is not excessive once it is realized that the evaluation is being conducted by the best people available. After all, the physician will be writing a clear-cut evaluation of the patient's status and making recommendations for care during the following year. This initial $500 examination is not unprecedented; it is similar in principle to having support coming only through qualified programs.

Now, coming to grips with the actual money to build. The proposed National Hemophilia Commission should be charged with finding sites for these 15 comprehensive centers. These sites should be selected on the basis of patient flow, accessibility, and the availability of expert staff. Selection, moreover, should be on the basis of competition. There may be great competition among those cities that have been chosen, for instance, in New York.

In addition to the 15 regional centers, there needs to be a satellite program. Although all care could be accomplished at regional centers, their physical location will deter some patients. The satellite program would be smaller centers that had been carefully evaluated and which now would be easily identified by their level of care--primary, secondary, or tertiary. A strategy to entice satellite centers into the system would be to offer to upgrade these satellite centers by using part of the $3 million allocated for building a regional center.

Education should occur at the satellite level. Education is vitally important. It is the only available means to ensure that the patient does not use his $5,000 just for prophylaxis. The hemophiliac must come to understand that his care includes more than just stocking his freezer with product.

With regard to research, the 10% allocation for research should go to the central facility for that region.

b. Education and Research Sector

First, not all the money should go into the bricks and mortar. Second, these regional centers should be associated with a university medical teaching school. Third, during the establishment of these centers, a plan for coordinating centers should be initiated. This plan should include: (1) a standardization of nomenclature; (2) guidelines to prohibit overlapping research and other activities; and (3) a centralized data collection system. We also strongly urge defining who is a hemophiliac. If this is not done, there will be a problem in determining the patient population. Fourth, the National Hemophilia Foundation's role would be to encourage members to use the centers. Also, the Foundation would use its resources to assist in casefinding to assure that centers are utilized fully.

Under this plan there would be $1.25 million for research per center of the $12.5 million total budget. Today only $4 million is allocated for hemophilia research. This new research money should be reserved for basic research emphasizing Factor VIII and IX molecular research. The technical research on blood products should be under the auspices of the regional blood banking system, which we have assumed is now in existence under the American Blood Commission (ABC). The centers should engage in research on health care delivery, testing the efficacy of home care, drug trials, etc. This type of clinical research should be paid for out of patient care dollars, not out of the 10% allocated for research.

No predetermined percentage should be designated for education and training. We assumed that Regional Blood Programs are operational;

these should interdigitate with hemophilia centers. There should be an educational director for continuing education programs for both professionals and paraprofessionals. From a budgetary standpoint, this should be the priority. Undergraduate education should be conducted through the teaching facility attached to the center. Lay and patient education should be a cooperative venture between centers and local NHF chapters.

Centers should establish their own evaluation mechanism in addition to any review and evaluation conducted by the universities associated with the centers and/or by the funding agency, in this case, the government. Also, the regional program for hemophilia must be in accord with the ABC regional blood banking program. We are concerned that the NIH hemophilia research dollar will lessen, but for now, we have assumed that there will be no reduction in hemophilia research.

c. Payment Sector

When the regulations are drawn up for this legislation, they should designate a lead agency to coordinate the government effort in such areas as finding sites and establishing quality control. This direction should emanate from the Office of the Assistant Secretary for Health. The grants procedure should follow the usual form.

As a mechanism for reimbursing these centers, we endorse using the existing machinery of the Bureau of Health Insurance. The Bureau is used now for some 20 million people, mainly Medicare and the disabled. This mechanism could be extended to cover 38,000 hemophiliacs. Entitlement for hemophiliacs would occur after the submission of medical information to the Bureau. The Bureau, then, would reimburse directly the physician, the institution, or the patient. To encourage patients to seek care from a comprehensive care center rather than from a private physician we have developed a two-tier plan. First, the Bureau of Health Insurance would pay the patient or his doctor whatever the physician charged. The Bureau would also pay any prescription charge. The second part of the plan would be to reimburse regional centers at the cost-related price, paying only for the actual cost of providing care--the audited cost. All research costs would be considered as part of the audited cost of care.

The patient's money should go farther in a center system than if he were receiving care from a private physician. In the centers, physicians would be salaried and there would be a high patient volume and economies of scale. Ultimately, there would be a lower unit cost. If this plan is implemented, it should stimulate patients to use centers. It should also counteract the nervousness of providers who are afraid centers will be under-utilized.

The payment group is nervous, however, about putting money into a system without affecting the delivery of service, the quality of care, or the efficiency of the system. The growth of the center system, therefore, should be gradual. Enough centers should be started to provide alternatives to existing care. Then, after two or three years of the parallel system, cost difference and quality system really was more efficient. Also, if the center system had been more efficient, this would provide the impetus to enlarge the center system.

d. Product Sector

We have approached the creation of these 15 regional centers in a number of ways. No matter how we approached it, we discovered a formidable roadblock. Our conception of a hemophilia center is that it is part of a regional network. Even though not every hemophiliac in that region would attend that center, its impact on care and treatment would result in a massive increase in demand. It is inevitable; the more care, the more surgery, and, then, the more demand for product.

This increased demand is the roadblock. In the interest of a blood system (both private and public) that is responsive and cost-effective, we must know what the demand will be. We need guidelines, but there are no guidelines. Can the experts tell us what the demand for Factors VIII and IX will be? What is the mix for severe and moderate hemophiliacs? For each level of care, home care, crisis care, propnylaxis, just how much product is required? Without this kind of information, there is simply no way we can plan our response rationally. The pharmaceutical industry will not know the upper limits of demand. It will not know if it should expand its plant capacity.

The product group recommends that all product be distributed in a planned manner, and by this we mean only through regional centers. If this is done, then it should be possible to conserve product, avoiding stockpiling by patients and distributing product according to need. For example, if there were a shortage of purified product, it could be saved and used only for those cases where its use is appropriate. It would be the reverse of what happens now which is that the patient who has enough money gets the best regardless if the best is what he needs. Implementing our recommendation would result in the more efficient use of material and would save money.

2. Floor Discussion

Product Sector: This 10% research allocation on the surface sounds like a very good idea, but is it? What about the quality of research, the need for peer review, accountability, and excellence? This must be examined critically.

Care Sector: It may never be a good idea to build into a system a set number of dollars for research that are not competed for. Every satellite could get 10% of the money for research and not do anything with it. The research money should be pooled and then be competed for.

Payment Sector: It is politically unrealistic to expect the patient to use his care dollar for research. Congress has said this $5,000 should go for care. It did not say this money should go for research.

Care Sector: The research dollar should be part of the blood delivery system, but Congress did not accept this. We have no reason to expect it will accept this in health care delivery. But without funds, no research will be carried out. There could be the "sky's the limit" as long as there is built in a mechanism for peer review. It is not money which has created the need for research. We need research to get the answers to obtain information. There is a need to know.

Product Sector: We accepted the establishment of hemophilia centers as though the money were in a lump sum. What really will happen is that there will be a competition. There will be start-up funds for research. But with competition means that someone wins. When the best is given three million, these will be centers of excellence and then there will be the others.

Payment Sector: Yes, but the payment mechanism will pay everyone. It will not destroy others. Still, it is true, centers and physicians will operate at different levels, but all will be paid. There will be a gradient, however.

Care Sector: That is why we proposed the satellite system. It would upgrade local care centers. If one really closely examines this proposed plan, one can see that there is no need for a facility. What is needed is an operating budget. We need funds to employ people who have the expertise to provide the care. At the moment, none of the programs are remunerative for their staff. They are volunteer staff. The question is, does the personnel exist to deliver the care? People make the program, not the plant.

Product Sector: To change the subject, we would like comment on this group's request for more information on how much product is needed. Last time, it was stated that 100 units of cryoprecipitate is needed by each hemophiliac per year. How much material is needed and what should the product mix be? How should it be controlled? While this is a game, these questions should be answered.

One way to introduce some control into the system is not to distribute blood products freely to every pharmacy, but to distribute them only to a regional center. There will not be absolute control, but it will permit the implementation of a rational plan.

Also, what about this fractionation issue that has been raised? What about creating a noncommercial fractionation plant?

Care Sector: Paying money from funds earmarked for hemophilia centers is like mixing apples and pears. Building a facility is not the same as care. The public ought to know this. There should be public accountability. They should know what their money is being spent on. A fractionation center should not be part of the care system.

Product Sector: The industry will not like the idea of a new fractionation center. To the industry, it will be like reinventing the wheel. Further, it may not be possible to set up a fractionation plant with concentrate as its main product. While research should occur outside of industry and more research should be occurring, it might be better to use this fractionation center as pilot project. Or, better still, ask the existing fractionation centers why they are not making products for hemophilia. Many are not making any.

On a philosophical basis, we should favor a fractionation center. The pharmaceutical industry's primary motivation is making money. But what if there is a product which, though necessary, is required by only a few and is expensive? This product should be made not in response to profit, but in response to need. This is very important.

Payment Sector: This fractionation center would compete gently with industry. No one would lose any business. In fact, if the fractionation center has access to pooled plasma and outdated blood, its product may be cheaper than industry which relies on plasmapheresis.

Product Sector: There should be a marriage between the regional blood center and the hemophilia center. It could be feasible in some places to connect regional blood centers to hemophilia centers.

B. PRESENTATION OF CONSEQUENCE A

You spent the morning thinking about and talking about the effects that an event would have on you and the ways it might alter your behavior. At the same time, though you couldn't see them and they didn't make a presentation here, others were viewing what happended during this first year, and we have another "News Bulletin" concerning that:

FIVE CENTERS NAMED TO CARRY ON HEMOPHILIA RESEARCH

"A panel of Department of Health, Education, and Welfare officials today designated five of the 15 National Hemophilia Centers as hemophilia research centers. The Department believes that cutting

back the number of centers engaged in research to five will avoid (1) the current duplication of effort and (2) the poor use of the research dollar. At the same time, concentrating research at five centers will stimulate research into those areas showing the most promise. All 15 centers will continue to offer comprehensive care, diagnostic testing, education, and training.

"The five centers were selected on the basis of their quality of research and proximity to other centers. As it stands now, 10% of each center's income must go for research in the center. Following today's announcement, the ten undesignated centers will phase out their research programs and transfer their research funds to a central pool to be proportioned among the five designated research centers. The five research centers are Chapel Hill, North Carolina; Chicago, Illinois; Houston, Texas; Los Angeles, California; and New York City, New York."

Group Statements by Participants

a. Product Sector

By taking away the research capacity of 10 of the 15 centers, 10 centers have been disenfranchised. Also, there will be a lower level of care. Not only might some research not be done, but the 10 non-research centers are now disadvantaged; they have had the stamp of "second class" placed on them.

A number of research areas will be affected including 100% detection and carrier detection, synthesis of the molecule, and *in vitro* production. Also, a large number of clinical investigations that need to be carried out may not be, including treatment of inhibitors, steroids as adjuncts to treatments, the effects of long-term plasma treatment, the optimal number of days that need postoperative product coverage, prophylaxis trials, liver disease complications studies, and more.

b. Payment Sector

This situation is not real. It is both unlikely and artificial. Using patient care money for research as it is described in this scenario would mean that the government is paying the patient the cost of care plus 10%. The government is by definition losing control over that 10%.

It does not correspond to how Congress appropriates money. For example, Medicare is not tied into research because it is impossible to fit research and care into the same appropriation. It is also a dangerous precedent to give a certain percentage for research. What will happen is that research dollars will be used

for demonstration and control programs instead of actual research. This research money should be given to the NIH which would permit these funds only to be used for actual research purposes.

c. Care Sector

Reducing the number of centers that can conduct research to five does not really concern us. What really worries us is whether good clinical research can be separated from good care.

d. Education and Research Sector

It is a terrible idea. Those 10 centers which have lost their research component will become pedestrian. Moreover, without a research program there will be inadequate training programs. Training at these 10 centers will be second-rate training. Also, there will be a raiding by the five centers for the best people in the 10 remaining centers.

Our group also does not like the way the money is to be divided equally among the five centers. We recommend, instead, that each center should request research proposals. The money should be granted on the basis of good research. Our group reiterates its position that research funds should be used only for basic, not clinical, research.

We acknowledge that there will never be true equality amongst the centers even with a most rigorous oversight committee. But, at the same time, inequality should not be perpetuated as this plan would do.

C. PRESENTATION OF CONSEQUENCE B

During the full time you have been meeting and discussing both events, yet another group has been viewing what has been going on, and we now have the last event for you to consider today. As soon as it's been read, please again prepare statements.

NATIONAL HEMOPHILIA CENTERS TO BECOME DEPOTS FOR BLOOD PRODUCTS

"To meet the hemophiliacs demand for plasma products and to obtain data on this demand, the Department of Health, Education, and Welfare has agreed to the American Blood Commission's request that the 15 National Hemophilia Centers become depots for all blood products used by hemophiliacs. Now, each center is required to dispense material by prescription to all hemophiliacs residing in the center's catchment area and/or using the center's facilities. Under this plan, the hemophilia center would be reimbursed directly for the products used by hemophiliacs. Physicians who treat

patients outside the center system would obtain their material from the hemophilia center with payment going directly to the center.

"A further DHEW stipulation is that each center make every effort to obtain blood products from those suppliers using voluntary donors. This policy also permits centers to negotiate directly with pharmaceutical firms to obtain the blood products at the lowest possible price. It is hoped that by centralizing blood product procurement that the price per unit will be more adequately controlled and that treaters will no longer be bidding against themselves for the available product."

1. Group Statements by Participants

a. Care Sector

The effects of having centers used as depots for products are different for those inside the center system than for those outside the system. Inside the system, there will be more control over the quality of care. The center patient should now have optimal care because product will be used correctly. The possibility also exists of using centralization of blood product to bring patients into the system. Some of us wanted restrictions on physician access to product, but there was no agreement. The individual physician outside the system is at risk. We recommend an impartial advisory board to assure equal physician access to product. Using centers as depots for product should not inhibit the use of product. The reverse should really happen. There will be more product and more should be quickly available because we will now know where it is. Product will no longer be at the mercy of a random system.

b. Education and Research Sector

Using the centers as depots for product will stress our continuing education programs to the limit. It will become critical to educate physicians about component therapy. What could result from this situation would be something like a narcotics board. A certain number of physicians are able to prescribe certain narcotics, once they have taken a certifying examination. Recertification should be on a yearly basis.

Having the product available at the centers will result in technical research coming to the fore. The centers by necessity will have to become familiar with management, storage and distribution of product. This is expensive and who is going to pay for the technical aspects of product management?

c. Payment Sector

This is a real situation. It is a mechanism which provides for easy control and which is cost effective. As to the education and research group's question on who would pay for the technical aspects of product management, the answer is third party payers would pay for all the costs incurred by centers in handling product. Third party payers would pay the audited cost. There would be no problem because all of these centers are nonprofit.

In New York, there already exists such an arrangement. The Greater New York Blood Program provides blood to all hospitals, and the hospitals receive free blood. In terms of central buying, all the procurement for blood is done by the program. The only problem with the program is that it lacks a good data collection system.

The question this group would like to raise is, how could this really happen? Could it happen as it is stated here, that HEW agrees to the request of the ABC?

d. Product Sector

This is certainly what the HEW intends to happen. It is true that the ABC does not have regulatory power, but it should come to the HEW saying these are what the ABC sees as desirable ends and to assure the attainment of these ends the HEW must issue regulations.

We propose channelling product not through hemophilia centers, but through regional blood centers, which should be in existence by this time. We make this recommendation because hemophilia centers are involved in care delivery and regional blood centers are involved in blood management. The regional blood centers have the expertise in storing, managing, and collecting data on blood.

Also, it is not necessary to have a legislative or executive mandate. In New York, the arrangement was worked out with the drug companies. The program began with only one drug company agreeing to the plan, and now all the other companies have joined. The drug companies like the arrangement because it means they do not need a sales force. It is advantageous for the hemophiliac as everything is in one place. The program obtains the product at a discount and this cheaper price is passed on to the hemophiliac. There should not be any legal problems to implementing such a policy.

2. Floor Discussion

Payment Sector: If there are any legal problems, there are ways around them. The regional blood authority could say that it would sell all its pooled plasma to manufacturers provided they sold back to the regional blood authority all the product the companies made. It is perfectly legal. Then there is no restraint of trade.

Education and Research Sector: In the past, the NHF has been approached and has been asked to distribute product to all hemophiliacs. The NHF has opposed this idea because it is a very large undertaking and the Foundation simply does not have the money to do it. The NHF does have a great interest in seeing centralized pharmacies come into being. The Foundation would recommend that product should not be allowed to be more centralized than a regional blood authority.

Product Sector: Perhaps the American Red Cross could implement this kind of policy. It has a policy of not doing business with commercial firms. But really, the Red Cross wears two faces. On the one hand, it gives its plasma to a commercial company to make product; while on the other hand, it refuses to sell it or promote it on a large scale. A Red Cross regional director could implement such a policy if he really wanted and if he were strong enough. As a matter of fact, there is nothing in the Red Cross regulations that specifically prohibits this. The problem for the Red Cross is its image. But if the public accepts this idea, then the Red Cross should have no problem putting it into effect.

Should the program have constraints on who prescribes material as the care group mentioned? We believe not. The centralization of product should be done without constraints, as a community service. Constraints would make the program unworkable.

Education and Research Sector: What about the hemophiliac himself? Little has been said about the rights of the consumer. What we have been saying is that what is right for the patient is right no matter what the patient wants. We are making the hemophiliacs a captive audience.

Payment Sector: We must realize that there is a social cost to the freedom of the individual. Can anyone smoke? What about the social cost of his smoking? It is the same thing for the hemophiliac. Think of the social cost of a crippled hemophiliac to our society. Does society have the right to say to him, you must get good care?

Education and Research Sector: We do not have the right to make him seek treatment because we do not know how to cure hemophilia. But if we did know and people were not availing themselves of this, then it might be different. We would have more of a right to insist that they seek treatment.

Care Sector: What is being said is that hemophiliacs are a captive audience. This is dangerous to the psychosocial well-being of the patient. Sometimes it's better to let the patient go right ahead and die; at least, he can do what he wants to do. Previously, it was possible to say to the hemophiliac, you must do this or you will die. Now, hemophilia is not that life-threatening.

Product Sector: But if he is crippled, he will be out of work and on welfare with society paying his medical costs. This is the social cost.

Education and Research Sector: But, that is the extreme. Another extreme is the crippled patient who has been crippled for a long time, and then is rehabilitated. If he wants to stay crippled in spite of rehabilitation, he will stay crippled.

Payment Sector: There are precedents for some kind of an accommodation to the social costs involved. But obviously, what was accommodation 10 or 20 years ago will not be an accommodation 25 years from now. Take people who are smoking themselves to death. Insurance companies are beginning to say no to people who are smoking. This change has occurred in about 10 years.

Education and Research Sector: But what if the hemophiliac wants to go to a certain doctor and we say, sorry you can't go to him, he is a bad doctor. Is this right?

Care Sector: A National Hemophilia Commission must begin to look at the level of care delivery, so that it is possible to say whether it is good or bad care. This is what is supposed to happen at the AMA grass roots level. We all know it is not happening there. That is why it is necessary to have a National Commission. We must begin to stand up and be counted and say that this care is good and that care is not good.

Payment Sector: Enforcing quality of care standards could be carried out by local Professional Standards and Review Organizations (PSRO). The PSRO could support good programs and not others. This would be especially effective if third party payers would reimburse only those programs which had been endorsed.

Education and Research Sector: We need a Hemophilia Commission independent of care because there is no one way to treat hemophilia. We do not really know how poor the care is really. If optimal care existed, then hemophiliacs would be up and walking around normally. Someone else may have a different idea of what optimal care is. The criteria are very controversial.

Product Sector: All of us are aware that someone who has an M.D. is more qualified to treat patients than someone who does not have this qualification. But if this is the level of care, then, indeed, we are aiming at the lowest delivery of care rather than at the highest.

Care Sector: The 1971 NHLI study found that many hemophiliacs are receiving care from a physician who is treating only one hemophiliac. Some of us are saying this is all right and others are saying this should be stopped.

Product Sector: Hemophilia care is not a matter of treating a patient once, it is not a one-time disease. This is a disease that lasts a lifetime and that is recurring and crippling. If in his childhood a hemophiliac is treated by a doctor who knows nothing about the disease, it is very likely that when this boy grows up he will be crippled. This is the difference in treatment.

Why not assemble 25 national leaders in hemophilia care and obtain a 25-doctor agreement on not what is acceptable in all circumstances, but 95% agreement on what the mode of therapy should be under most conditions. Where there is 95% agreement draw up a list of questions that someone who is treating one or two hemophiliacs must answer correctly before he can continue to treat these patients. This could be enforced by exercising control over payment. He would not be reimbursed with Federal money if he could not answer the quiz.

Payment Sector: There is another system for payment in New York which works according to this principle. The State Department of Health made a decision that it would pay only for certain services in certain hospitals and in its crippled children's program. The State examined the hospitals and said it would pay for arthritis care at one hospital and not others, and cardiac care here but not there. The payers have worked to upgrade the system. This is what we must do.

Care Sector: We should be asking ourselves, where are we going to be in five or ten years time? Can we identify good versus bad systems of care? Then, if we recognize a system as bad, shouldn't we do something about it? Are some of us saying no to this?

Education and Research Sector: But we are also saying that we have become an authority, that is, we know how to treat hemophilia. But a good educator teaches his medical students to be prepared for 20 years hence, rather than turning to some impressive board stating this is how it should be.

Moderator: We are arguing over how to regulate society. This is an important issue. We have identified a series of issues. How do you build excellence into the system? How do you organize and stabalize medical care practice?

Appendix B

GLOSSARY OF SELECTED TERMS

Appendix B

GLOSSARY OF SELECTED TERMS

Clotting Factors	Proteins, in solution in plasma, that contribute to the coagulation process
Component of Blood	Product separated from blood by physical means. Thus, cryoprecipitated Factor VIII is a blood component, being prepared through physical means of centrifugation, freezing, and thawing.
Cryoprecipitate	Component of blood containing high concentration of Factor VIII which is used in replacement therapy for classic hemophilia. Prepared from a single-donor unit of plasma by centrifugation under proper freezing and thawing conditions, yielding a 3-ml paste containing as much as 50% of the original Factor VIII. Cryoprecipitate does not contain Factor IX.
Dry Concentrates	Blood products refined to powdered form through chemical manipulation which are reconstituted with sterile water for infusion.
(1) Factor VIII Dry Concentrate	Dried concentrate of Factor VIII, used in treating classic hemophilia.
(2) Dried Prothrombin Complex	Dried concentrate of several clotting factors (Factor IX and Factors II, VII, and X), which is used in treating Factor IX deficiency.
Episodic Care	Treatment, i.e., infusion of blood product, in response to an episode of bleeding. Included under "episodic care" is "aggresive" episodic care, or the infusion at the first suspicion of a bleeding episode.
Factor VIII	(AHF, Antihemophilic Factor). A protein in solution in plasma, essential to the intrinsic clotting mechanism. Deficiency of Factor VIII results in classic hemophilia. Blood products containing Factor VIII are infused as therapy for classic hemophilia.

Factor IX (PTC)	A protein in solution in plasma, essential to the intrinsic clotting mechanism. Deficiency of Factor IX results in Christmas disease (Hemophilia B). Blood products containing Factor IX are infused as therapy for Hemophilia B.
Fraction of Blood	Product produced by chemical manipulation of blood and/or blood components. Dry concentrates used in replacement therapy of Hemophilia A & B are fractions, unlike cryoprecipitated Factor VIII.
Fresh-Frozen plasma	Plasma which has been frozen within six hours of collection; when thawed, contains approximately 80% of the original amounts of clotting factors.
Hemophilia	A hereditary blood disorder in which the deficiency of a factor in the blood prevents normal coagulation, resulting in excessive external and internal bleeding.
Hemophilia A	Deficiency of Factor VIII, one of the clotting factors essential in the intrinsic blood clotting mechanism. Also called classic hemophilia, and Factor VIII deficiency.
Hemophilia B	Deficiency of Factor IX, another clotting factor essential in the intrinsic clotting mechanism. Also called Christmas disease and Factor IX deficiency.
Home Care	Infusion of the blood product by the patient or his family, instead of by a clinician, thus allowing patient to receive replacement therapy at home or while traveling; requires storage of product at home. Home care or "self-infusion" can be applicable to both episodic care and prophylactic care.
Mild Hemophilia	Generally detected only after severe trauma or surgery during which bleeding cannot easily be controlled. By assay, circulative level of the clotting factor ranges from 10% to 30% of normal. Mild hemophiliacs (as judged by their physicians) were excluded from the survey.

Moderate hemophilia	Clinically, rarely hemorrhages spontaneously, but may experience significant hemorrhage after mild trauma. By assay, the circulative level of the clotting factor ranges from 1% to 10% of normal.
Plasma	The fluid remaining when cells have been removed from whole blood. Clotting factors, including Factor VIII and Factor IX, are in solution in plasma.
Prophylaxis (preventive care)	Infusion of blood product to raise and maintain the amount of clotting factor at a specified level to forestall episode(s) of bleeding, as distinct from episodic care, in which bleeding occurs or is suspected before infusion is begun.
Replacement Therapy	The basis of treating the hemorrhagic aspects of hemophilia; intravenous infusion of blood product containing the deficient clotting factor to raise the circulating level of the factor.
Severe Hemophilia	Clinically, the patient is subject to hemorrhage arising from no apparent cause (spontaneous), as well as bleeding after any type of trauma or minor surgery. By assay, the circulating level of the deficient clotting factor is less than 1% of normal.
Severity	Three general categories to denote the degree of each patient's deficiency of the clotting factor: (1) severe, (2) moderate, (3) mild. Severity is judged in two principal ways: (a) by clinical manifestation--the physician judges the patient's severity according to the clinical symptoms presented; (b) by assay of the patient's blood, to determine the amount of clotting factor present in the plasma. NOTE: In this survey, data regarding severity are based on the best judgment of physicians sampled. Some physicians based their classifications on assayed circulating levels; others on clinical evaluation. There was minimal deviation between clinical and assay categorization

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	of severe hemophilia since the spontaneous bleeding by which severe hemophilia is identified occurs only when the circulating level is less than 1% of normal. The delineation between moderate and mild is less distinct.
Treaters	Physicians treating the bleeding aspects of hemophilia.
Unit of Cryoprecipitate (bag)	The amount of cryoprecipitate recovered in past form from one bag of plasma. A bag of cryoprecipitate contains an average of about 100 to 120 units of AHF, and contains no PTC (Factor IX).
Unit of Factor VIII or AHF	The amount of AHF activity found in 1 ml of "normal" plasma. Therefore, one bag of plasma with 238 ml would contain 238 units of AHF.
Unit of Factor IX or PTC	Amount of PTC activity found in 1 ml of "normal" plasma.
Unit of Whole Blood	500 ml ("pint") containing 432 ml whole blood from a single donor and 68 ml anticoagulant. One pint of "normal" whole blood contains about 238 AHF units and 238 PTC units at collection.

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